

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

☒ QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(D) OF THE SECURITIES EXCHANGE ACT OF 1934

For the quarterly period ended March 31, 2025

☐ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(D) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from _____ to _____

Commission File Number 001-39267

BENITEC BIOPHARMA INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

84-462-0206
(IRS Employer
Identification No.)

3940 Trust Way, Hayward, California 94545
(Address of principal executive offices & zip code)

(510) 780-0819
(Registrant's telephone number including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.0001	BNTC	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐

Accelerated filer ☐

Non-accelerated filer ☒

Smaller reporting company ☒

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ or No ☒

We had 25,600,210 shares of our common stock outstanding as of the close of business on May 14, 2025.

BENITEC BIOPHARMA INC.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Report contains forward-looking statements that are subject to a number of risks and uncertainties, many of which are beyond our control. Our forward-looking statements relate to future events or our future performance and include, but are not limited to, statements concerning our business strategy, future commercial revenues, market growth, capital requirements, new product introductions, expansion plans and the adequacy of our funding. All statements, other than statements of historical fact included in this Report, are forward-looking statements. When used in this Report, the words “could,” “believe,” “anticipate,” “intend,” “estimate,” “expect,” “may,” “continue,” “predict,” “potential,” “project,” or the negative of these terms, and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain such identifying words. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements.

Some of the risks and uncertainties that may cause our actual results, performance or achievements to differ materially from those expressed or implied by forward-looking statements include the following:

- the success of our plans to develop and potentially commercialize our product candidates;
- the timing of the completion of preclinical studies and clinical trials;
- the timing and sufficiency of patient enrollment and dosing in any future clinical trials;
- the timing of the availability of data from our clinical trials;
- the timing and outcome of regulatory filings and approvals;
- the development of novel AAV vectors;
- our potential future out-licenses and collaborations;
- the plans of licensees of our technology;
- the clinical utility and potential attributes and benefits of ddRNAi and our product candidates, including the potential duration of treatment effects and the potential for a “one shot” cure;
- our intellectual property position and the duration of our patent portfolio;
- expenses, ongoing losses, future revenue, capital needs and needs for additional financing, and our ability to access additional financing given market conditions and other factors, including our capital structure;
- the length of time over which we expect our cash and cash equivalents to be sufficient to execute on our business plan;
- unanticipated delays;
- further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development;
- the ability to enroll sufficient numbers of subjects in clinical trials;
- determinations made by the U.S. Food and Drug Administration and other governmental authorities;
- regulatory developments in the United States of America;
- our ability to protect and enforce our patents and other intellectual property rights;
- our dependence on our relationships with our collaboration partners and other third parties;
- the efficacy or safety of our products and the products of our collaboration partners;
- the acceptance of our products and the products of our collaboration partners in the marketplace and market competition;
- sales, marketing, manufacturing and distribution requirements;
- greater than expected expenses, expenses relating to litigation or strategic activities;
- the impact of, and our ability to remediate, the identified material weakness in our internal controls over financial reporting;

-
- our ability to satisfy our capital needs through increasing revenue and obtaining additional financing; and
 - the impact of local, regional and national and international economic conditions and events;

as well as other risks detailed under the caption “Risk Factors” in this Report and in other reports filed with the SEC. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Report, we caution you that these statements are based on a combination of facts and important factors currently known by us and our expectations of the future, about which we cannot be certain. Such statements are based on assumptions and the actual outcome will be affected by known and unknown risks, trends, uncertainties and factors that are beyond our control or ability to predict. We have based the forward-looking statements included in this Report on information available to us on the date of this Report or on the date thereof. Except as required by law we undertake no obligation to revise or update any forward-looking statements, whether as a result of new information, future events or otherwise. You are advised to consult any additional disclosures that we may make directly to you or through reports that we, in the future, may file with the SEC, including annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K.

All forward-looking statements included herein or in documents incorporated herein by reference are expressly qualified in their entirety by the cautionary statements contained or referred to elsewhere in this Report.

PART I—FINANCIAL INFORMATION

ITEM 1. Financial Statements

BENITEC BIOPHARMA INC.
Consolidated Balance Sheets
(in thousands, except par value and share amounts)

	March 31, 2025 (Unaudited)	June 30, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 103,583	\$ 50,866
Restricted cash	63	63
Trade and other receivables	3	229
Prepaid and other assets	361	516
Total current assets	104,010	51,674
Property and equipment, net	145	179
Deposits	55	25
Prepaid and other assets	35	62
Right-of-use assets	964	270
Total assets	<u>\$ 105,209</u>	<u>\$ 52,210</u>
Liabilities and stockholders' equity		
Current liabilities:		
Trade and other payables	\$ 6,254	\$ 4,165
Accrued employee benefits	426	475
Lease liabilities, current portion	346	284
Total current liabilities	7,026	4,924
Non-current accrued employee benefits	—	38
Lease liabilities, less current portion	613	—
Total liabilities	<u>7,639</u>	<u>4,962</u>
Stockholders' equity:		
Preferred stock, \$0.0001 par value—5,000,000 shares authorized; no shares issued and outstanding at March 31, 2025 and June 30, 2024, respectively	—	—
Common stock, \$0.0001 par value—160,000,000 shares authorized; 25,546,288 shares and 10,086,119 shares issued and outstanding at March 31, 2025 and June 30, 2024, respectively	2	1
Additional paid-in capital	310,313	238,398
Accumulated deficit	(212,029)	(190,259)
Accumulated other comprehensive loss	(716)	(892)
Total stockholders' equity	<u>97,570</u>	<u>47,248</u>
Total liabilities and stockholders' equity	<u>\$ 105,209</u>	<u>\$ 52,210</u>

The accompanying notes are an integral part of these consolidated financial statements.

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BENITEC BIOPHARMA INC.
Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended March 31,		Nine Months Ended March 31,	
	2025	2024	2025	2024
Revenue:				
	\$ —	\$ —	\$ —	\$ —
Total revenues	—	—	—	—
Operating expenses				
Royalties and license fees	—	(3)	—	(108)
Research and development	5,980	2,566	14,637	12,097
General and administrative	4,208	1,578	9,952	4,953
Total operating expenses	10,188	4,141	24,589	16,942
Loss from operations	(10,188)	(4,141)	(24,589)	(16,942)
Other income (loss):				
Foreign currency transaction gain (loss)	11	(118)	(190)	(22)
Interest income (expense), net	823	(4)	2,250	(16)
Other expense, net	—	(16)	(5)	(50)
Gain on extinguishment of liabilities	—	—	764	—
Unrealized loss on investment	—	—	—	(1)
Total other income (loss), net	834	(138)	2,819	(89)
Net loss	\$ (9,354)	\$ (4,279)	\$ (21,770)	\$ (17,031)
Other comprehensive income:				
Unrealized foreign currency translation gain (loss)	(28)	117	176	(5)
Total other comprehensive income (loss)	(28)	117	176	(5)
Total comprehensive loss	\$ (9,382)	\$ (4,162)	\$ (21,594)	\$ (17,036)
Net loss	\$ (9,354)	\$ (4,279)	\$ (21,770)	\$ (17,031)
Deemed dividends	—	—	—	(619)
Net loss attributable to common shareholders	\$ (9,354)	\$ (4,279)	\$ (21,770)	\$ (17,650)
Net loss per share:				
Basic and diluted	\$ (0.24)	\$ (0.23)	\$ (0.63)	\$ (1.11)
Weighted average number of shares outstanding: basic and diluted	38,599,453	18,281,896	34,559,870	15,876,753

The accompanying notes are an integral part of these consolidated financial statements.

BENITEC BIOPHARMA INC.
Consolidated Statements of Stockholders' Equity
(Unaudited)
(in thousands, except share amounts)

	Common Stock		Additional	Accumulated	Accumulated	Total
	Shares	Amount	Paid-in	Deficit	Other Comprehensive	Stockholders'
			Capital		Loss	Equity
Balance at June 30, 2023	1,671,485	\$ —	\$168,921	\$ (167,889)	\$ (830)	\$ 202
Issuance of common stock, pre-funded warrants, and common warrants sold for cash, net of offering costs of \$2,964	875,949	—	27,919	—	—	27,919
Anti-dilution adjustment to warrants	—	—	619	(619)	—	—
Share-based compensation	—	—	91	—	—	91
Foreign currency translation gain	—	—	—	—	50	50
Net loss	—	—	—	(5,954)	—	(5,954)
Balance at September 30, 2023	2,547,434	—	197,550	(174,462)	(780)	22,308
Exercise of pre-funded warrants	25,000	—	—	—	—	—
Exercise of Series 2 warrants	20,000	—	39	—	—	39
Share-based compensation	—	—	93	—	—	93
Foreign currency translation loss	—	—	—	—	(172)	(172)
Net loss	—	—	—	(6,798)	—	(6,798)
Balance at December 31, 2023	2,592,434	\$ —	\$197,682	\$ (181,260)	\$ (952)	\$ 15,470
Exercise of pre-funded warrants	132,360	—	—	—	—	—
Share-based compensation	—	—	192	—	—	192
Foreign currency translation loss	—	—	—	—	117	117
Net loss	—	—	—	(4,279)	—	(4,279)
Balance at March 31, 2024	2,724,794	\$ —	\$197,874	\$ (185,539)	\$ (835)	\$ 11,500
Balance at June 30, 2024	10,086,119	\$ 1	\$238,398	\$ (190,259)	\$ (892)	\$ 47,248
Exercise of pre-funded warrants	1,768,454	—	—	—	—	—
Exercise of Series 2 warrants	857,845	—	1,655	—	—	1,655
Exercise of common warrants	5,181,347	—	20,002	—	—	20,002
Share-based compensation	—	—	435	—	—	435
Foreign currency translation loss	—	—	—	—	(101)	(101)
Net loss	—	—	—	(5,059)	—	(5,059)

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	Common Stock		Additional	Accumulated	Accumulated	Total
	Shares	Amount	Paid-in	Deficit	Other Comprehensive Loss	Stockholders' Equity
Balance at September 30, 2024	17,893,765	1	260,490	(195,318)	(993)	64,180
Exercise of pre-funded warrants	606,129	—	—	—	—	—
Exercise of Series 2 warrants	642,160	—	1,240	—	—	1,240
Exercise of common warrants, net of issuance costs of \$2	4,309,421	—	16,630	—	—	16,630
Share-based compensation	—	—	943	—	—	943
Foreign currency translation gain	—	—	—	—	305	305
Net loss	—	—	—	(7,357)	—	(7,357)
Balance at December 31, 2024	23,451,475	\$ 1	\$279,303	\$ (202,675)	\$ (688)	\$ 75,941
Issuance of common stock and pre-funded warrants sold for cash, net of offering costs of \$2,245	2,043,000	1	28,212	—	—	28,213
Exercise of common warrants	51,813	—	201	—	—	201
Share-based compensation	—	—	2,597	—	—	2,597
Foreign currency translation gain	—	—	—	—	(28)	(28)
Net loss	—	—	—	(9,354)	—	(9,354)
Balance at March 31, 2025	25,546,288	\$ 2	\$310,313	\$ (212,029)	\$ (716)	\$ 97,570

The accompanying notes are an integral part of these consolidated financial statements.

BENITEC BIOPHARMA INC.
Consolidated Statements of Cash Flows
(Unaudited)
(in thousands)

	Nine Months Ended March 31,	
	2025	2024
Cash flows from operating activities:		
Net loss	\$ (21,770)	\$(17,031)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	52	61
Amortization of right-of-use assets	226	191
Unrealized loss on investment	—	1
Gain on extinguishment of liabilities	(764)	—
Share-based compensation expense	3,975	376
Changes in operating assets and liabilities:		
Trade and other receivables	226	—
Prepaid and other assets	147	1,051
Trade and other payables	2,862	(602)
Accrued employee benefits	(82)	48
Lease liabilities	(247)	(205)
Net cash used in operating activities	(15,375)	(16,110)
Cash flows from investing activities:		
Purchase of property and equipment	(18)	(179)
Net cash used in investing activities	(18)	(179)
Cash flows from financing activities:		
Proceeds from issuance of common stock, pre-funded warrants, and common warrants	30,459	30,883
Proceeds from exercise of pre-funded warrants, Series 2 warrants common warrants	39,729	39
Share and pre-funded warrants issuance transaction costs	(2,247)	(2,964)
Net cash provided by financing activities	67,941	27,958
Effects of exchange rate changes on cash, cash equivalents, and restricted cash	169	(3)
Net increase in cash, cash equivalents, and restricted cash	52,717	11,666
Cash, cash equivalents, and restricted cash, beginning of period	50,929	2,490
Cash, cash equivalents, and restricted cash, end of period	<u>\$103,646</u>	<u>\$ 14,156</u>
Supplemental disclosure of cash flow information		
Initial measurement of operating lease right-of-use assets and liabilities	\$ 255	\$ —
Re-measurement of operating lease right-of-use assets and liabilities	\$ 666	\$ —
Deemed dividend	\$ —	\$ 619

The accompanying notes are an integral part of these consolidated financial statements.

BENITEC BIOPHARMA INC.
Notes to Consolidated Financial Statements
(Unaudited)

1. Business

Benitec Biopharma Inc. (the “Company”, “we”, “our”) is a corporation formed under the laws of Delaware, United States of America, on November 22, 2019 and listed on the Nasdaq Capital Market (“Nasdaq”) under the symbol “BNTC”. Benitec Biopharma Inc. is the parent entity of a number of subsidiaries including the previous parent entity Benitec Biopharma Limited (“BBL”). BBL was incorporated under the laws of Australia in 1995 and was listed on the Australian Securities Exchange, or ASX, from 1997 until April 15, 2020. On August 14, 2020, BBL reorganized as a Proprietary Limited company and changed its name to Benitec Biopharma Proprietary Limited. The Company’s business focuses on the development of novel genetic medicines. Our proprietary platform is called “Silence and Replace” DNA-directed RNA interference. The proprietary “Silence and Replace” DNA-directed RNA interference platform combines RNA interference, or RNAi, with gene therapy to create medicines that simultaneously facilitate sustained silencing of disease-causing genes and concomitant delivery of wildtype replacement genes following a single administration of the therapeutic construct.

During the year ended June 30, 2021, the Company completed an organization restructuring as part of the commercial desire to provide a more efficient structure for the future as the Company transitioned its operations to the United States.

The Company’s fiscal year end is June 30. References to a particular “fiscal year” are to our fiscal year end June 30 of that calendar year.

The consolidated financial statements of Benitec Biopharma Inc. are presented in United States dollars and consist of Benitec Biopharma Inc. and its wholly owned subsidiaries as listed below. Aside from Benitec Biopharma Proprietary Limited, the international subsidiaries are dormant.

	Principal place of business/country of incorporation
Benitec Biopharma Proprietary Limited (“BBL”)	Australia
Benitec Australia Proprietary Limited	Australia
Benitec Limited	United Kingdom
Benitec, Inc.	USA
Benitec LLC	USA
RNAi Therapeutics, Inc.	USA
Tacere Therapeutics, Inc.	USA
Benitec IP Holdings, Inc.	USA

2. Basis of Presentation and Summary of Significant Accounting Policies

Basis of Presentation

The Company’s consolidated financial statements contained in this Quarterly Report on Form 10-Q have been prepared in accordance with generally accepted accounting principles in the U.S. (“GAAP”) for interim financial information and with the instructions to Form 10-Q and Article 8 of U.S. Securities and Exchange Commission (“SEC”) Regulation S-X. Accordingly, certain information and disclosures required by GAAP for annual financial statements have been omitted. In the opinion of management, all adjustments, consisting of normal recurring adjustments, considered necessary for a fair presentation have been included. Interim financial results are not necessarily indicative of results anticipated for the full year. These consolidated financial statements should be read in conjunction with the Company’s audited financial statements and accompanying notes included in the Company’s Annual Report on Form 10-K for the year ended June 30, 2024.

Reference is frequently made herein to the Financial Accounting Standards Board (the “FASB”) Accounting Standards Codification (“ASC”). This is the source of authoritative GAAP recognized by the FASB to be applied to non-governmental entities.

Principles of Consolidation

The consolidated financial statements include the Company’s accounts and the accounts of its wholly owned subsidiaries. All intercompany transactions and balances have been eliminated.

Use of Estimates

The preparation of the Company’s consolidated financial statements requires management to make estimates and assumptions that impact the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in the Company’s consolidated financial statements and accompanying notes. The most significant estimates and assumptions in the Company’s consolidated financial statements relate to accrued research and development expense and valuation of equity-based instruments issued for other than cash. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company’s future results of operations will be affected.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, protection of proprietary technology, dependence on key personnel, reliance on single-

source vendors and collaborators, availability of raw materials, patentability of the Company's products and processes and clinical efficacy and safety of the Company's products under development, compliance with government regulations and the need to obtain additional financing to fund operations.

There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained or maintained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid technological change and substantial competition from other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, consultants and other third parties.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business in one operating segment.

Foreign Currency Translation and Other Comprehensive Income (Loss)

The Company's functional currency and reporting currency is the United States dollar. BBL's functional currency is the Australian dollar (AUD). Assets and liabilities are translated at the exchange rate in effect at the balance sheet date. Revenues and expenses are translated at the average rate of exchange prevailing during the reporting period. Equity transactions are translated at each historical transaction date spot rate. Translation adjustments arising from the use of different exchange rates from period to period are included as a component of stockholders' equity as "Accumulated other comprehensive loss." Gains and losses resulting from foreign currency translation are included in the consolidated statements of operations and comprehensive loss as other comprehensive income (loss).

Other comprehensive income (loss) for all periods presented consists entirely of foreign currency translation gains and losses.

Fair Value Measurements

The Company measures its financial assets and liabilities in accordance with GAAP using ASC 820, *Fair Value Measurements*. For certain financial instruments, including cash and cash equivalents, accounts receivable, and accounts payable, the carrying amounts approximate fair value due to their short maturities.

The Company follows accounting guidance for financial assets and liabilities. ASC 820 defines fair value, provides guidance for measuring fair value and requires certain disclosures. The guidance utilizes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value into three broad levels. The following is a brief description of those three levels:

- Level 1: Observable inputs such as quoted prices (unadjusted) in active markets for identical assets or liabilities.
- Level 2: Inputs, other than quoted prices that are observable, either directly or indirectly. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active.
- Level 3: Unobservable inputs in which little or no market data exists, therefore developed using estimates and assumptions developed by us, which reflect those that a market participant would use.

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Cash and Cash Equivalents

Cash and cash equivalents include cash on hand and at banks, short-term deposits with an original maturity of three months or less with financial institutions. There were no cash equivalents as of March 31, 2025 and June 30, 2024.

Restricted cash balances of \$63 thousand and \$63 thousand as of March 31, 2025 and June 30, 2024, respectively, are used to secure the Company's credit card.

Concentrations of Risk

Financial instruments that potentially subject the Company to significant concentration of credit risk consist primarily of cash. The Company maintains deposits at federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts, and management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

Trade and Other Receivables

The Company adopted ASC 326—*Financial Instruments—Credit Losses (Topic 326)* as of July 1, 2023. As such, the Company estimates current expected credit losses (CECL) on trade and other receivables on an ongoing basis, and will recognize those expected credit losses immediately. Estimates of current expected credit losses will be based on analyses of individual customer circumstances and historical write-off experience. The Company's analyses will consider the aging of receivable accounts, customer creditworthiness, and general economic conditions.

Property and Equipment

Property and equipment are stated at cost, net of accumulated depreciation and amortization. Expenditures for maintenance and repairs are expensed as incurred; additions, renewals, and improvements are capitalized. When property and equipment are retired or otherwise disposed of, the related cost and accumulated depreciation and amortization are removed from the respective accounts, and any gain or loss is included in operations. Depreciation and amortization of property and equipment is calculated using the straight-line basis over the following estimated useful lives:

Software	3-4 years
Lab equipment	3-7 years
Computer hardware	3-5 years

Impairment of Long-Lived Assets

Property and equipment are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of long-lived assets to be held and used is measured by a comparison of the carrying amount of an asset to the estimated undiscounted future cash flows expected to be generated by the asset. If the carrying amount of an asset exceeds its estimated undiscounted future cash flows, an impairment charge is recognized by the amount by which the carrying amount of the asset exceeds the fair value of the assets. Fair value is generally determined using the asset's expected future discounted cash flows or market value, if readily determinable.

Trade and other payables

These amounts represent liabilities for goods and services provided to the Company prior to the end of the period and which are unpaid. Due to their short-term nature, they are measured at cost and are not discounted. The amounts are unsecured and are usually paid within 30 days of recognition.

Leases

At lease commencement, the Company records a lease liability based on the present value of lease payments over the expected lease term. The Company calculates the present value of lease payments using the discount rate implicit in the lease, unless that rate cannot be readily determined. In that case, the Company uses its incremental borrowing rate, which is the rate of interest that the Company would have to pay to borrow on a collateralized basis an amount equal to the lease payments over the expected lease term. The Company records a corresponding right-of-use lease asset based on the lease liability, adjusted for any lease incentives received and any initial direct costs paid to the lessor prior to the lease commencement date.

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After lease commencement, the Company measures its leases as follows: (i) the lease liability based on the present value of the remaining lease payments using the discount rate determined at lease commencement; and (ii) the right-of-use lease asset based on the remeasured lease liability, adjusted for any unamortized lease incentives received, any unamortized initial direct costs and the cumulative difference between rent expense and amounts paid under the lease agreement. Any lease incentives received and any initial direct costs are amortized on a straight-line basis over the expected lease term. Rent expense is recorded on a straight-line basis over the expected lease term.

Lease terms may include options to extend or terminate the lease when the Company is reasonably certain that it will exercise the option. Certain lease agreements may contain variable costs such as utilities and common area maintenance. Variable lease costs are expensed when the cost is incurred.

The Company elected the short-term lease practical expedient that allows entities to recognize lease payments on a straight-line basis over the lease term for leases with a term of 12 months or less. The Company has also elected the practical expedient under ASC Topic 842 allowing entities to not separate non-lease components from lease components, but instead account for such components as a single lease component for all leases.

Basic and Diluted Net Loss Per Share

Basic net loss per share is calculated by dividing net loss by the weighted-average number of common shares outstanding during the period. Diluted net loss per share is calculated by dividing net loss by the weighted-average number of common shares outstanding plus potential common shares. Stock options, warrants and convertible instruments are considered potential common shares and are included in the calculation of diluted net loss per share using the treasury stock method when their effect is dilutive. Potential common shares are excluded from the calculation of diluted net income (loss) per share when their effect is anti-dilutive. As of March 31, 2025, and June 30, 2024, there were 25,799,817 and 35,453,286 potential common shares, respectively, that were excluded from the calculation of diluted net loss per share because their effect was anti-dilutive.

Correction of Immaterial Error

During the third quarter of 2025, the Company identified an immaterial error in the Company's previously issued consolidated financial statements related to weighted-average number of common shares outstanding within the net loss per share computation. The error pertains to the exclusion of pre-funded warrants from the weighted-average number of common shares used in the computation of net loss per share. The Company assessed materiality, including qualitative and quantitative factors, and determined the error is immaterial to both the current and prior periods. The Company has revised the comparative net loss per share information as presented and disclosed within these consolidated financial statements. The revision had no effect on the Consolidated Balance Sheet, Consolidated Statements of Cash Flows, Consolidated Statements of Stockholders' Equity, or to reported net losses.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606—*Revenue from Contracts with Customers* ("ASC 606"). The core principle of ASC 606 is that entities are to recognize revenue to depict the transfer of promised goods and services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services.

The Company recognizes revenue in accordance with that core principle by applying the following steps: Step 1: Identify the contract(s) with a customer.

Step 2: Identify the performance obligations in the contract.

Step 3: Determine the transaction price.

Step 4: Allocate the transaction price to the performance obligations in the contract.

Step 5: Recognize revenue when (or as) the entity satisfies a performance obligation.

The Company applies judgement in determining whether contracts entered into fall within the scope of ASC 606. In doing so, management considers the commercial substance of the transaction and how risks and benefits of the contract accrue to the various parties to the contract.

Management has also made the judgement that the grant of the license and transfer of associated know-how and materials are accounted for as one performance obligation as they are not considered to be distinct; they are highly interrelated and could not provide benefits to the customer independently from each other. Judgements were made in relation to the transfer of the license and know-how and whether this should be recognized over time or a point in time. The point in time has been determined with regard to the point at which the transfer of know-how has substantially been completed and the customer has control of the asset and the ability to direct the use of and receive substantially all of the remaining benefits.

Licensing revenues

Revenue from licensees of the Company's intellectual property reflects the transfer of a right to use the intellectual property as it exists at the point in time in which the license is transferred to the customer. Consideration can be variable and is estimated using the most likely amount method and is constrained to the extent that it is probable that a significant reversal will not occur. Revenue is recognized as or when the performance obligations are satisfied.

The Company recognizes contract liabilities for consideration received in respect of unsatisfied performance obligations and reports these amounts as other liabilities in the consolidated balance sheet. Similarly, if the Company satisfies a performance obligation before it receives the consideration, the Company recognizes either a contract asset or a receivable in its consolidated balance sheet, depending on whether something other than the passage of time is required before the consideration is due.

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Royalties

Revenue from licensees of the Company's intellectual property reflect a right to use the intellectual property as it exists at the point in time in which the license is granted. Where consideration is based on sales of product by the licensee, revenue is recognized when the customer's subsequent sales of products occur.

Services revenue

Revenue is earned (constrained by variable considerations) from the provision of research and development services to customers. Services revenue is recognized when performance obligations are either satisfied over time or at a point in time. Generally, the provision of research and development services under a contract with a customer will represent satisfaction of a performance obligation over time where the Company retains the right to payment for services performed but not yet completed.

Research and Development Expense

Research and development expenses relate primarily to the cost of conducting clinical and pre-clinical trials. Pre-clinical and clinical development costs are a significant component of research and development expenses. The Company records accrued liabilities for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of pre-clinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and includes these costs in trade and other payables on the consolidated balance sheets and within research and development expenses on the consolidated statements of operations and comprehensive loss.

The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance at the end of each reporting period. As actual costs become known, the Company adjusts its accrued liabilities. The Company has not experienced any material differences between accrued costs and actual costs incurred.

Share-based Compensation Expense

The Company records share-based compensation in accordance with ASC 718, *Stock Compensation*. ASC 718 requires the fair value of all share-based compensation awarded to employees and non-employees to be recorded as an expense over the shorter of the service period or the vesting period. The Company determines employee and non-employee share-based compensation based on the grant-date fair value using the Black-Scholes Option Pricing Model.

Common Stock Warrants

The Company classifies certain warrants for the purchase of shares of its common stock as equity on its consolidated balance sheets as these warrants are considered indexed to the Company's shares of Common Stock. For warrants that do not meet the criteria of a liability warrant and are classified on the Company's consolidated balance sheets as equity instruments, the Company uses the Black-Scholes model to measure the value of the warrants at issuance.

Income Taxes

The Company is subject to Australia and United States income tax laws. The Company follows ASC 740, *Accounting for Income Taxes*, when accounting for income taxes, which requires an asset and liability approach to financial accounting and reporting for income taxes. Deferred income tax assets and liabilities are computed annually for temporary differences between the financial statements and tax bases of assets and liabilities that will result in taxable or deductible amounts in the future based on enacted tax laws and rates applicable to the periods in which the differences are expected to affect taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amount more likely than not to be realized. For uncertain tax positions that meet a "more likely than not" threshold, the Company recognizes the benefit of uncertain tax positions in the consolidated financial statements. The Company's practice is to recognize interest and penalties, if any, related to uncertain tax positions in income tax expense in the consolidated statements of operations.

Recent Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13: *Financial Instruments—Credit Losses (Topic 326)*. This ASU represents a significant change in the accounting for credit losses model by requiring immediate recognition of management's estimates of current expected credit losses (CECL). Under the prior model, losses were recognized only as they were incurred. The Company adopted this ASU effective July 1, 2023 and determined that its impact on the accompanying consolidated financial statements is immaterial.

Recently Issued Accounting Standards Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740) – Improvements to Income Tax Disclosures*, which enhances the transparency, effectiveness, and comparability of income tax disclosures by requiring consistent categories and greater disaggregation of information related to income tax rate reconciliations and the jurisdictions in which income taxes are paid. This guidance is effective for annual periods beginning after December 15, 2024 with early adoption permitted. The Company is currently evaluating the impact of the ASU on its income tax disclosures within the consolidated financial statements.

In November 2024, the FASB issued ASU 2024-03, ASC 220- Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures, which requires entities, in the notes to financial statements, to disclose specified information about certain costs and expenses. The guidance is effective for the Company’s annual periods beginning after December 15, 2026, and interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is assessing the impact of adopting this guidance on its consolidated financial statements.

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In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280) – Improvements to Reportable Segment Disclosures*, which improves reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. This ASU also expands disclosure requirements to enable users of financial statements to better understand the entity's measurement and assessment of segment performance and resource allocation. This guidance is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The Company is currently evaluating the impact of the ASU on its disclosures within the consolidated financial statements.

3. Liquidity

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. For the nine months ended March 31, 2025, and 2024, the Company incurred net losses of \$21.8 million and \$17.0 million, respectively, and used cash in operations of \$15.4 million and \$16.1 million, respectively. The Company expects to continue to incur additional operating losses in the foreseeable future.

The Company's business focuses on the development of novel genetic medicines and, at this stage in the Company's development, the Company has not established a source of revenue to cover its full operating costs, and as such, is dependent on funding operations through capital financing activities. As of March 31, 2025, the Company had \$103.6 million in cash and cash equivalents. On April 22, 2024 we closed a private investment in public equity (PIPE) financing in which we sold 5,749,152 shares of common stock at a price per share of \$4.80 and, in lieu of shares of common stock, pre-funded warrants to purchase up to an aggregate of 2,584,239 shares of common stock at a price per pre-funded warrant of \$4.7999, to certain accredited institutional investors. The pre-funded warrants were immediately exercisable until exercised in full at an exercise price of \$0.0001 per share of common stock. Gross proceeds from the financing totaled \$40.0 million. The Company also received additional cash during the nine months ended March 31, 2025 due to warrant exercises, warrant issuances, and common stock issuances totaling \$70.2 million. See Note 9. Stockholders Equity. We estimate that our cash and cash equivalents will be sufficient to fund the Company's operations for at least the next twelve months from the date of this report.

The Company's ability to continue as a going concern is dependent upon its ability to manage its net loss, become profitable, and obtain adequate financing. While the Company believes in its ability to generate revenue and raise additional funds, there can be no assurances to that effect. The financial statements do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that might be necessary if the Company is unable to continue as a going concern due to unsuccessful product development or commercialization, or the inability to obtain adequate financing in the future.

4. Cash, cash equivalents, and restricted cash

(US\$'000)	March 31, 2025	June 30, 2024
Cash at bank	\$103,583	\$50,866
Restricted cash	63	63
Total	<u>\$103,646</u>	<u>\$50,929</u>

5. Prepaid and other assets

(US\$'000)	March 31, 2025	June 30, 2024
Prepaid expenses	\$ 395	\$ 577
Market value of listed shares	1	1
Total other assets	396	578
Less: non-current portion	(35)	(62)
Current portion	<u>\$ 361</u>	<u>\$ 516</u>

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6. Property and equipment, net

(US\$'000)	March 31, 2025	June 30, 2024
Software	\$ 6	\$ 6
Lab equipment	1,533	1,521
Computer hardware	32	32
Furniture and fixtures	6	—
Leasehold improvements	24	24
Total property and equipment, gross	1,601	1,583
Accumulated depreciation and amortization	(1,456)	(1,404)
Total property and equipment, net	\$ 145	\$ 179

Depreciation and amortization expense was \$13 thousand and \$52 thousand for the three and nine months ended March 31, 2025, and \$25 thousand and \$61 thousand respectively, for the same periods in 2024.

7. Trade and other payables

(US\$'000)	March 31, 2025	June 30, 2024
Trade payable	\$ 433	\$1,351
Accrued consultant fees	36	75
Accrued professional fees	394	97
Accrued clinical development project costs	5,234	2,504
Other payables	157	138
Total	\$ 6,254	\$4,165

During the nine months ended March 31, 2025, the Company agreed to resolve disputed trade payables and accrued clinical development project costs of \$1.2 million with a vendor for \$495 thousand. This settlement resulted in a gain of \$764 thousand recorded in the statement of operations and other comprehensive loss under other income (expense) for the period.

8. Leases

The Company has entered into two operating leases for office spaces as of March 31, 2025. On February 1, 2025, the Company entered into a new lease which has an initial expiration date in 2026. On February 24, 2025, the Company entered into an amendment to an existing lease to extend the lease expiration date to 2027 and modify the remaining lease payments. The lease modification was not accounted for as a separate contract and instead the existing operating lease right-of-use asset and liability were remeasured during the period under agreements that expire in 2026 and 2027. Both leases contain options to extend for additional renewal periods. The leases require the Company to pay utilities, insurance, taxes, and other operating expenses. The Company's lease does not contain any residual value guarantees or material restrictive covenants.

The tables below show the changes during the nine months ended March 31, 2025:

(US\$'000)	Operating lease right- of- use assets
Balance at July 1, 2024	\$ 270
Re-measurement during the period	666
Initial measurement at February 1, 2025	254
Amortization of right of use asset	(226)
Operating lease right-of-use asset at March 31, 2025	\$ 964

(US\$'000)	Operating lease liabilities
Balance at July 1, 2024	\$ 284
Re-measurement during the period	666
Initial measurement at February 1, 2025	255
Principal payments on operating lease liabilities	(246)
Operating lease liabilities at March 31, 2025	959
Less: non-current portion	(613)
Current portion at March 31, 2025	\$ 346

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As of March 31, 2025, the Company's operating leases have a weighted average lease term of 2.67 years and a weighted average discount rate of 6%. The leases' options to extend are not included within the remaining lease term as the Company is currently not reasonably certain to exercise such options. The maturities of the operating lease liabilities are as follows:

(US\$'000)	March 31, 2025
2025	\$ 267
2026	435

2027	331
Total operating lease payments	1,033
Less imputed interest	(75)
Present value of operating lease liabilities	\$ 959

The Company recorded lease liabilities and right-of-use lease assets for the lease based on the present value of lease payments over the expected lease term, discounted using the Company's incremental borrowing rate. The incremental borrowing rate was determined based on quoted rates by the Company's business banker for collateralized debt with terms similar to the lease agreements. Rent expense was \$0.1 million and \$0.2 million for the three and nine months ended March 31, 2025, respectively, and \$0.1 million and \$0.2 million for the same periods in 2024, and is reported within general and administrative on the consolidated statements of operations and comprehensive loss.

9. Stockholders' equity

Preferred Stock

On December 6, 2024, the stockholders of the Company approved an amendment (the "Amendment") to the Company's Amended and Restated Certificate of Incorporation, as amended, to authorize the issuance of 5,000,000 shares of preferred stock, par value \$0.0001. As of March 31, 2025, there were no preferred shares issued and outstanding.

Common Stock

On December 8, 2021, the stockholders of the Company approved an amendment (the "Charter Amendment") to the Company's Amended and Restated Certificate of Incorporation to increase the total number of authorized shares of common stock of the Company from 10,000,000 to 40,000,000, which became effective on December 17, 2021. On December 7, 2022, the stockholders of the Company approved another amendment to the Company's Amended and Restated Certificate of Incorporation to increase the number of authorized shares of common stock from 40,000,000 to 160,000,000. The Charter Amendment was filed with the Secretary of State of the State of Delaware and became effective December 9, 2022. On July 26, 2023, the Company effected a 1-for-17 reverse stock split (the "Reverse Stock Split").

On October 11, 2024, the Company entered into a Sales Agreement (the "Sales Agreement") with Leerink Partners LLC (the "Agent"). Pursuant to the terms of the Sales Agreement, the Company may offer and sell shares of the Company's common stock having an aggregate offering amount of up to \$75 million from time to time through the Agent. The Agent will use its commercially reasonable efforts, as the agent and subject to the terms of the Sales Agreement, to sell the shares offered. Sales of the shares, if any, may be made in sales deemed to be an "at-the-market offering" as defined in Rule 415 under the Securities Act of 1933, as amended. The Company may also agree to sell shares to the Agent as principal for its own account on terms agreed to by the Company and the Agent. The Agent will be entitled to a commission from the Company of 3.0% of the gross proceeds from the sale of shares sold under the Sales Agreement. In addition, the Company has agreed to reimburse certain expenses incurred by the Agent in connection with the offering.

Concurrently with the 2025 Underwritten Offering (as defined below), on March 25, 2025, the Company also entered into a Securities Purchase Agreement to which the Company issued and sold 900,000 shares of Common Stock in a registered direct offering at a purchase price of \$13.00 per share. Gross proceeds from registered direct offering was \$11.7 million less underwriter issuance costs of \$0.7 million. The Company entered into a registration rights agreement in connection with the closing of the registered direct offering. The agreement required the Company to use its best efforts to register the shares for resale no later than 60 days following the closing of the registered direct offering.

Warrants and Common Stock

On December 6, 2019, investors were issued four Purchase Warrants that were exercisable into 12,600 fully paid shares of common stock should the Purchase Warrants be exercised in full ("Purchase Warrants"). The exercise price for the Purchase Warrants is \$178.50 per share issued on exercise of a Purchase Warrant. The Purchase Warrants are exercisable, in whole or in part, any time from the date of issue until the fifth anniversary of the date of issue (December 6, 2024). On April 22, 2020, the Company issued 2,201 shares of common stock in connection with a cashless exercise of Purchase Warrants exercisable for 6,300 shares of common stock. The Company did not have an effective registration statement registering the resale of the Warrant Shares by the Holder at the time the Holder wanted to exercise the warrant; therefore, the Holder carried out a cashless exercise. The formula for conducting a cashless exercise was outlined in the Warrant agreement. 6,300 purchase warrants remained unexercised and expired in December 2024.

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On September 15, 2022, we closed an underwritten public offering in which we issued and sold (i) 1,037,520 shares of the Company's common stock, (ii) 12,171,628 pre-funded warrants, which, after giving effect to the Reverse Stock Split, are currently exercisable into 715,979 shares of common stock at an exercise price of \$0.0017 per share until exercised in full and (iii) 29,809,471 Series 2 warrants (the "Series 2 Warrants"), which, after giving effect to the Reverse Stock Split, are currently exercisable into 1,753,503 shares of common stock at an exercise price of \$11.22 per share. The Series 2 warrants sold in the offering became exercisable commencing December 9, 2022, the date on which the Company had both (a) received approval from its stockholders to increase the number of shares of common stock it is authorized to issue and (b) effected such stockholder approval by filing with the Secretary of State of the State of Delaware a certificate of amendment to its Amended and Restated Certificate of Incorporation, and will expire on the fifth anniversary of such initial exercise date. The combined purchase price for each share of common stock and accompanying common warrant was \$10.20, which was allocated as \$10.03 per share of common stock and \$0.17 per common warrant. The Series 2 Warrants contain an exercise price adjustment mechanism providing that certain issuances of common stock (or common stock equivalents), if made at a price lower than the then existing exercise price of such Series 2 Warrants would reset the exercise price to such lower price. As a result of the August 11, 2023 public offering, the exercise price of the Series 2 Warrants has been automatically reset as of the closing time of such public offering to \$1.9299. For the nine months ended March 31, 2024, we recorded a deemed dividend, for accounting purposes, during the fiscal quarter ended September 30, 2024 of \$618,987 as a result of an adjustment to the exercise price of its Series 2 Warrants due to an exercise price adjustment provision in such warrants.

On October 17, 2022 and October 27, 2022, investors exercised 117,939 and 9,804 pre-funded warrants, respectively, at an exercise price of \$0.0017 per share.

On August 11, 2023 we closed an underwritten public offering in which we sold 875,949 shares of common stock, 15,126,226 pre-funded warrants to purchase 15,126,226 shares of common stock, and 16,002,175 common warrants to purchase up to 16,002,175 shares of common stock. The combined purchase price for each share of common stock and accompanying common warrant was \$1.93, which was allocated as \$1.9299 per share of common

stock and \$0.0001 per common warrant. Each pre-funded warrant was sold together with one common warrant at a combined price of \$1.9299, which was allocated as \$1.9298 per pre-funded warrant and \$0.0001 per common warrant. The pre-funded warrants were immediately exercisable until exercised in full at an exercise price of \$0.0001 per share of common stock. The common warrants were immediately exercisable at an exercise price of \$3.86 per share of common stock and will expire on the fifth anniversary of such initial exercisable date. In addition, the Company granted the underwriter a 30-day option to purchase up to 2,331,606 additional shares of common stock and/or up to 2,331,606 additional common warrants. The underwriter partially exercised this option and purchased 458,134 additional shares of common stock and 458,134 additional common warrants. These additional shares are included in the total sold on August 11, 2023. Net proceeds from the offering, including the impact of the underwriter's partial exercise of its option and net of underwriting discounts, commissions, and other offering expenses, totaled \$27.9 million.

On October 17, 2023 an investor exercised 25,000 pre-funded warrants at an exercise price of \$0.0001 per share. On November 24, 2023, an investor exercised 20,000 Series 2 warrants at an exercise price of \$1.93 per share. On March 15, 2024 and March 18, 2024, investors exercised 105,888 and 26,472 pre-funded warrants, respectively, at an exercise price of \$0.0001 per share.

On April 10, 2024 and April 19, 2024, investors exercised 25,000 Series 2 warrants on each date, at an exercise price of \$1.93 per share. On April 22, 2024, an investor exercised 28,039 Series 2 warrants at an exercise price of \$1.93 per share. On April 23, 2024, May 8, 2024, and May 21, 2024, investors exercised 27,500, 697,475, and 13,212 common warrants, respectively, at an exercise price of \$3.86 per share.

On April 22, 2024 we closed a private investment in public equity (PIPE) financing in which we sold 5,749,152 shares of common stock at a price per share of \$4.80 and, in lieu of shares of common stock, pre-funded warrants to purchase up to an aggregate of 2,584,239 shares of common stock at a price per pre-funded warrant of \$4.7999, to certain accredited institutional investors. The pre-funded warrants were immediately exercisable until exercised in full at an exercise price of \$0.0001 per share of common stock. Gross proceeds from the financing totaled \$40.0 million.

Net proceeds, net of commissions and other offering expenses, totaled approximately \$37.1 million.

On April 26, 2024, April 28, 2024, and May 16, 2024, investors exercised 350,000, 438,000, and 7,947 pre-funded warrants, respectively, at an exercise price of \$0.0001 per share.

On July 25, 2024, an investor exercised 269,609 Series 2 warrants at an exercise price of \$1.93 per share. On September 12, 2024, an investor exercised 200,000 pre-funded warrants at an exercise price of \$0.0001 per share.

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On August 29, 2024, the Company's stockholders approved the exercise of certain existing warrants issued in April 2024, September 15, 2022 and August 11, 2023 in accordance with the rules of the Nasdaq Stock Market which otherwise would be subject to the Beneficial Ownership Limitation.

On September 26, 2024, investors exercised 1,368,180 pre-funded warrants at an exercise price of \$0.0001 per share and exercised 5,181,347 common warrants at an exercise price of \$3.86 per share. Also on September 26, 2024, an investor exercised 588,236 Series 2 warrants at an exercise price of \$1.93 per share. On September 27, 2024, an investor exercised 200,274 pre-funded warrants on a cashless basis.

On October 15, 2024, an investor exercised 425,000 pre-funded warrants at an exercise price of \$0.0001 per share. On October 15, 2024, October 17, 2024, October 18, 2024, and October 29, 2024, investors exercised 1,496,214, 308,803, 950,000, 1,554,404 common warrants, respectively, at an exercise price of \$3.86 per share. Also on October 29, 2024, an investor exercised 588,239 Series 2 warrants at an exercise price of \$1.93 per share.

On December 12, 2024, an investor exercised 181,129 pre-funded warrants on a cashless basis. Also on December 18, 2024, investors exercised 53,921 Series 2 warrants at an exercise price of \$1.93 per share.

On February 25, 2025, an investor exercised 51,813 common warrants, at an exercise price of \$3.86 per share.

On March 25, 2025, the Company entered into an underwriting agreement to which the Company issued and sold (i) 1,143,000 shares of the Company's common stock, par value \$0.0001 per share at a purchase price to investors of \$13.00 per share, and (ii) pre-funded warrants to purchase 300,000 shares of Common Stock at an exercise price of \$0.0001 per share at a purchase price to investors of \$12.999 per warrant. Total gross proceeds from underwriting offering (the "2025 Underwritten Offering") was \$18.8 million less underwriter issuance costs of \$1.1 million and other cash issuance costs of \$0.4 million. The pre-funded warrants are exercisable immediately and do not have an expiration date.

Total net proceeds received by the Company during the nine-month period ended March 31, 2025 from the issuance of common stock, prefunded warrants, and exercises of warrants totaled \$67.9 million.

As of March 31, 2025, there were 21,147,677 warrants outstanding.

The activity related to warrants for the nine months ended March 31, 2025, is summarized as follows:

	Common Stock from Warrants	Weighted- average Exercise Price (per share)
Outstanding at July 1, 2024	34,271,146	\$ 1.85
Pre-funded warrants exercised	1,768,454	\$0.0001
Series 2 warrants exercised	857,845	\$1.9299
Common warrants exercised	5,181,347	\$ 3.86
Outstanding and exercisable at September 30, 2024	26,463,500	\$ 1.57
Pre-funded warrants exercised	606,129	\$0.0001
Series 2 warrants exercised	642,160	\$1.9299
Common warrants exercised	4,309,421	\$ 3.86
Purchase warrants expired	6,300	\$178.50
Outstanding and exercisable at December 31, 2024	20,899,490	\$ 1.08
Pre-funded warrants issued March 25, 2025	300,000	\$0.0001

Common warrants exercised	51,813	\$ 3.86
Outstanding and exercisable at March 31, 2025	21,147,677	\$ 1.06

Equity Incentive Plan

Employee Share Option Plan

In connection with its re-domiciliation to the United States, the Company assumed BBL's obligations with respect to the settlement of options that were issued by BBL prior to the re-domiciliation pursuant to the Benitec Officers' and Employees' Share Option Plan (the "Plan"). This includes the Company's assumptions of the Plan and all award agreements pursuant to which each of the options were granted. Each option when exercised entitles the option holder to one share in the Company. Options are exercisable on or before an

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expiry date, do not carry any voting or dividend rights and are not transferable except on death of the option holder or in certain other limited circumstances. Employee options vest one third on each anniversary of the applicable grant date for three years. If an employee dies, retires, or otherwise leaves the organization, and certain other conditions have been satisfied, generally the employee has 12 months to exercise their options, or the options are cancelled. After the Re-domiciliation, no new options have been or will be issued under the Plan.

On July 1, 2024, the Plan and all options granted thereunder expired by its and their terms.

Equity and Incentive Compensation Plan

On December 9, 2020, the Company's stockholders approved the Company's 2020 Equity and Incentive Compensation Plan (the "2020 Plan"). The 2020 Plan provides for the grant of various equity awards. Currently, only stock options are outstanding under the 2020 Plan. Each option when exercised entitles the option holder to one share of the Company's common stock. Options are exercisable on or before an expiry date, do not carry any voting or dividend rights, and are not transferable except on death of the option holder or in certain other limited circumstances. Employee stock options vest in increments of one-third on each anniversary of the applicable grant date over three years. Non-employee director options vest in increments of one-third on the day prior to each of the Company's next three annual stockholder meetings following the grant date. Executive Options granted on December 9, 2024, and December 27, 2024, vest in sixteen substantially equal quarterly installments on the last day of each full fiscal quarter of the Company ending after the grant date. If an option holder dies or terminates employment or service due to Disability (as defined in the 2020 Plan), the option holder generally has 12 months to exercise their vested options, or the options are cancelled. If an option holder otherwise leaves the Company, other than for a termination by the Company for Cause (as defined in the 2020 Plan), the option holder generally has 90 days to exercise their vested options, or the options are cancelled. The maximum contractual term of options granted under the 2020 Plan is ten years. Upon the consummation of a Change in Control (as defined in the 2020 Plan), all unvested stock options will immediately vest as of immediately prior to the Change in Control.

On December 8, 2021, the Company's stockholders approved an amendment to the 2020 Plan, which increased the number of shares of the Company's common stock reserved under the 2020 Plan to 108,823 (as adjusted for the Reverse Stock Split). For the fiscal year ended June 30, 2024, our named executive officers ("NEO's") were each granted equity incentive awards under the 2020 Plan. On December 6, 2023, the Company's stockholders approved an amendment to the 2020 Plan, which increased the number of shares of the Company's common stock reserved under the 2020 Plan to 1,204,537. On August 29, 2024, the Company's stockholders approved an amendment to the 2020 Plan, which increased the number of shares of the Company's common stock reserved under the 2020 Plan to 8,204,537.

Equity Awards

The activity related to equity awards, which are comprised of stock options during the nine months ended March 31, 2025 is summarized as follows:

	Stock Options	Weighted-average Exercise Price	Weighted-average Remaining Contractual Term	Aggregate Intrinsic Value
Outstanding at June 30, 2024	1,182,140	\$ 6.58	9.51 years	\$ 2,342,847
Granted	3,470,000	12.16	9.94 years	1,615,500
Expired	—	—	—	—
Forfeited	—	—	—	—
Outstanding at March 31, 2025	4,652,140	\$ 10.75	9.46 years	\$12,160,372
Exercisable at March 31, 2025	628,669	\$ 10.42	8.95 years	\$ 3,258,553

Share-Based Compensation Expense

The classification of share-based compensation expense is summarized as follows:

(US\$'000)	Three Months Ended		Nine Months Ended	
	March 31,		March 31,	
	2025	2024	2025	2024
Research and development	\$ 302	\$ 45	\$ 538	\$ 112
General and administrative	2,295	147	3,437	264
Total share-based compensation expense	\$ 2,597	\$ 192	\$ 3,975	\$ 376

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As of March 31, 2025, there was \$37.4 million of unrecognized share-based compensation expense related to stock options issued under the Share Option Plan and the 2020 Plan, which is expected to be recognized over a weighted average period of 3.52 years.

10. Income taxes

For the three and nine months ended March 31, 2025, and March 31, 2024, respectively, the Company did not recognize a provision or benefit for income taxes as it has incurred net losses. In addition, the net deferred tax assets generated from net operating losses are fully offset by a valuation allowance as the Company believes it is not more likely than not that the benefit will be realized.

11. Commitments and contingencies

Contract commitments

The Company enters into contracts in the normal course of business with third-party contract research organizations, contract development and manufacturing organizations and other service providers and vendors. These contracts generally provide for termination on notice and, therefore, are cancellable contracts and not considered contractual obligations and commitments.

Contingencies

From time to time, the Company may become subject to claims and litigation arising in the ordinary course of business. The Company is not a party to any material legal proceedings, nor is it aware of any material pending or threatened litigation.

12. Related party transactions

During the three and nine month ended March 31, 2025 and March 31, 2024, the Company did not enter into any related party transactions.

13. Subsequent events

On April 11, 2025, an investor exercised 53,922 Series 2 warrants at an exercise price of \$1.93 per share.

You should read the following discussion and analysis of financial condition and operating results together with our consolidated financial statements and the related notes and other financial information included elsewhere in this document.

Company Overview

We endeavor to become the leader in discovery, development, and commercialization of therapeutic agents capable of addressing significant unmet medical need via the application of the silence and replace approach to the treatment of genetic disorders.

Benitec Biopharma Inc. ("Benitec" or the "Company" or in the third person, "we" or "our") is a clinical-stage biotechnology company focused on the advancement of novel genetic medicines with headquarters in Hayward, California. The proprietary platform, called DNA-directed RNA interference, or ddRNAi, combines RNA interference, or RNAi, with gene therapy to create medicines that facilitate sustained silencing of disease-causing genes following a single administration. The unique therapeutic constructs also enable the simultaneous delivery of wildtype replacement genes, facilitating the proprietary "silence and replace" approach to the treatment of genetically defined diseases. The Company is developing a silence and replace-based therapeutic (BB-301) for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD), a chronic, life-threatening genetic disorder.

BB-301 is a silence and replace-based genetic medicine currently under development by Benitec. BB-301 is an AAV-based gene therapy designed to permanently silence the expression of the disease-causing gene (to slow, or halt, the biological mechanisms underlying disease progression in OPMD) and to simultaneously replace the mutant gene with a wildtype gene (to drive restoration of function in diseased cells). This fundamental therapeutic approach to disease management is called "silence and replace." The silence and replace mechanism offers the potential to restore the normative physiology of diseased cells and tissues and to improve treatment outcomes for patients suffering from the chronic, and potentially fatal, effects of OPMD. BB-301 has been granted Orphan Drug Designation in the United States and the European Union.

The targeted gene silencing effects of RNAi, in conjunction with the durable transgene expression achievable via the use of modified viral vectors, imbues the silence and replace approach with the potential to produce permanent silencing of disease-causing genes along with simultaneous replacement of the wild type gene function following a single administration of the proprietary genetic medicine. We believe that this novel mechanistic profile of the current and future investigational agents developed by Benitec could facilitate the achievement of robust and durable clinical activity while greatly reducing the frequency of drug administration traditionally expected for medicines employed for the management of chronic diseases. Additionally, the achievement of permanent gene silencing and gene replacement may significantly reduce the risk of patient non-compliance during the course of medical management of potentially fatal clinical disorders.

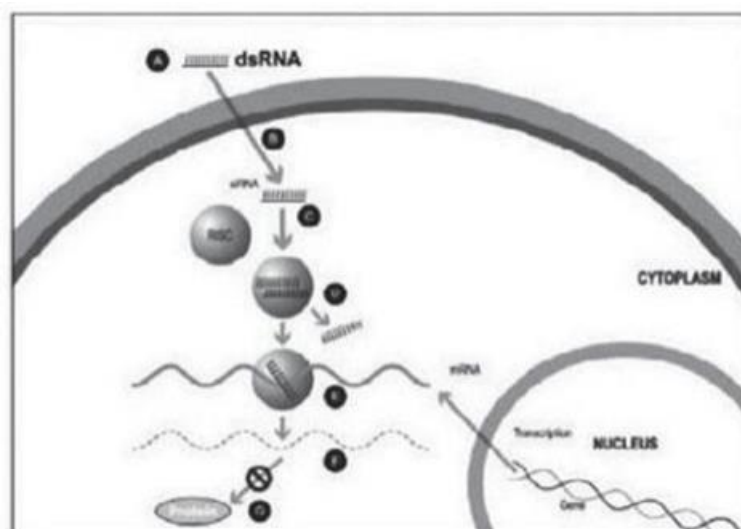
We will require additional financing to progress our product candidates through to key inflection points.

Our proprietary technology platforms are designated as DNA-directed RNA interference, or "ddRNAi", and "silence and replace." ddRNAi is designed to produce permanent silencing of disease-causing genes, by combining RNA interference, or RNAi, with viral delivery agents typically associated with the field of gene therapy (i.e., viral vectors). Modified AAV vectors are employed to deliver genetic constructs which encode short hairpin RNAs that are, then, serially expressed and processed to produce siRNA molecules within the transduced cell for the duration of the life of the target cell. These newly introduced siRNA molecules drive permanent silencing of the expression of the disease-causing gene. The silence and replace approach further bolsters the biological benefits of permanent silencing of disease-causing genes by incorporating multifunctional genetic constructs within the modified AAV vectors to create an AAV-based gene therapy agent that is designed to silence the expression of disease-causing genes (to slow, or halt, the underlying mechanism of disease progression) and to simultaneously replace the mutant genes with normal, "wildtype" genes (to drive restoration of function in diseased cells). This fundamentally distinct therapeutic approach to disease management offers the potential to restore the underlying physiology of the treated tissues and, in the process, improve treatment outcomes for patients suffering from the chronic and, potentially, fatal effects of diseases like Oculopharyngeal Muscular Dystrophy (OPMD).

Traditional gene therapy is defined by the introduction of an engineered transgene to correct the pathophysiological derangements derived from mutated or malfunctioning genes. Mutated genes can facilitate the intracellular production of disease-causing proteins or hamper the production of critical, life-sustaining, proteins. The introduction of a new transgene can facilitate the restoration of production of normal proteins within the diseased cell, thus restoring natural biological function. Critically, the implementation of this traditional method of gene therapy cannot eliminate the expression, or the potential deleterious effects of, the underlying mutant gene (as mutant proteins may be continually expressed and aggregate or drive the aggregation of other native proteins within the diseased cell). In this regard, the dual capabilities of the proprietary silence and replace approach to silence a disease-causing gene via ddRNAi and simultaneously replace the wild type activity of a mutant gene via the delivery of an engineered transgene could facilitate the development of differentially efficacious treatments for a range of genetic disorders.

The mutation of a single gene can cause a chronic disease via the resulting intracellular production of a disease-causing protein (i.e., an abnormal form of the protein of interest), and many chronic and/or fatal disorders are known to result from the inappropriate expression of a single gene or multiple genes. In some cases, genetic disorders of this type can be treated exclusively by “silencing” the intracellular production of the disease-causing protein through well-validated biological approaches like RNA interference (“RNAi”). RNAi employs small nucleic acid molecules to activate an intracellular enzyme complex, and this biological pathway temporarily reduces the production of the disease-causing protein. In the absence of the disease-causing protein, normal cellular function is restored and the chronic disease that initially resulted from the presence of the mutant protein is partially or completely resolved. RNAi is potentially applicable to over 20,000 human genes and a large number of disease-causing microorganism-specific genes.

Figure 1



A small double stranded RNA, or dsRNA, molecule (A, Figure 1), comprising one strand known as the sense strand and another strand known as the antisense strand, which are complementary to each other, is synthesized in the laboratory. These small dsRNAs are called small interfering RNAs, or siRNAs. The sequence of the sense strand corresponds to a short region of the target gene mRNA. The siRNA is delivered to the target cell (B, Figure 1), where a group of enzymes, referred to as the RNA-Induced Silencing Complex, or RISC, process the siRNA (C, Figure 1), where one of the strands (usually the sense strand) is released (D, Figure 1). RISC uses the antisense strand to find the mRNA that has a complementary sequence (E, Figure 1) leading to the cleavage of the target mRNA (F, Figure 1). As a consequence, the output of the mRNA (protein production) does not occur (G, Figure 1). Several companies, including Alnylam Pharmaceuticals Inc. (“Alnylam”), utilize this approach in their RNAi product candidates.

Importantly, many genetic disorders are not amenable to the traditional gene silencing approach outlined in Figure 1, as the diseased cells may produce a mixture of the wild type protein of interest and the disease-causing mutant variant of the protein, and the underlying genetic mutation may be too small to allow for selective targeting of the disease-causing variant of the protein through the use of siRNA-based approaches exclusively. In these cases, it is extraordinarily difficult to selectively silence the disease-causing protein without simultaneously silencing the wild type intracellular protein of interest whose presence is vital to the conduct of normal cellular functions.

Our proprietary silence and replace technology utilizes the unique specificity and robust gene silencing capabilities of RNAi while overcoming many of the key limitations of siRNA-based approaches to disease management.

In the standard RNAi approach, double-stranded siRNA is produced synthetically and, subsequently, introduced into the target cell via chemical modification of the RNA or alternative methods of delivery. While efficacy has been demonstrated in several clinical indications through the use of this approach, siRNA-based approaches maintain a number of limitations, including:

- Clinical management requires repeat administration of the siRNA-based therapeutic agent for multiple cycles to maintain efficacy;
- Long-term patient compliance challenges due to dosing frequencies and treatment durations;
- Therapeutic concentrations of siRNA are not stably maintained because the levels of synthetic siRNA in the target cells decrease over time;
- Novel chemical modifications or novel delivery materials are typically required to introduce the siRNA into the target cells, making it complicated to develop a broad range of therapeutics agents;
- Potential adverse immune responses, resulting in serious adverse effects;
- Requirement for specialized delivery formulations for genetic disorders caused by mutations of multiple genes; and
- siRNA acts only to silence genes and cannot be used to replace defective genes with normally functioning genes.

Our Approach to the Treatment of Genetic Diseases—ddRNAi and Silence and Replace

Our proprietary silence and replace approach to the treatment of genetic diseases combines RNAi with wild type gene replacement to drive permanent silencing of disease-causing genes and concomitant restoration of functional wild type genes following a single administration of the therapeutic agent. Benitec employs ddRNAi in combination with classical gene therapy (i.e., transgene delivery via viral vectors) to overcome several of the fundamental limitations of RNAi.

The silence and replace approach to the treatment of genetic disorders employs adeno-associated viral vectors (“AAVs”) to deliver genetic constructs which may, after a single administration to the target tissues:

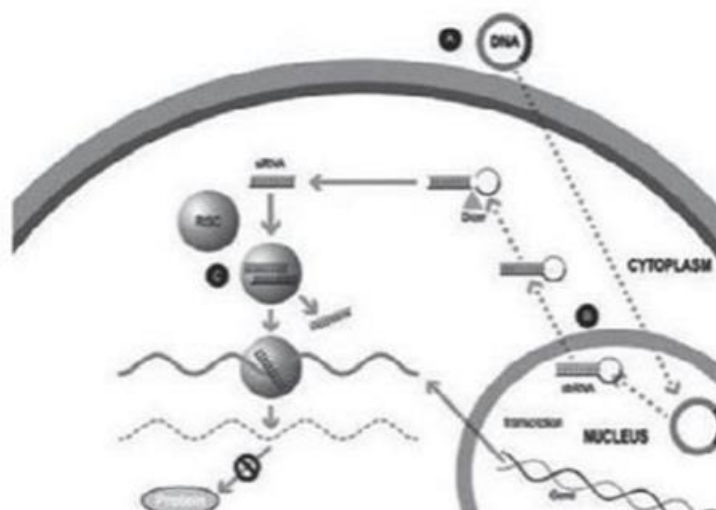
- Chronically express RNAi molecules inside of the target, diseased, cells (to serially silence the intracellular production of mutant, disease-causing, protein and the wild type protein of interest);
- Simultaneously drive the expression of a wild type variant of the protein of interest (to restore native intracellular biological processes); and
- AAV vectors can accommodate the multi-functional DNA expression cassettes containing the engineered wild type transgenes and the novel genes encoding short hairpinRNA/microRNA molecules (shRNA/miRNA) that are required to support the development of therapeutic agents capable of the achievement of the goals of the silence and replace approach to therapy.

Our silence and replace technology utilizes proprietary DNA expression cassettes to foster continuous production of gene silencing shRNAs and wild type proteins (via expression of the wild type transgene). A range of viral gene therapy vectors can be used to deliver the DNA construct into the nucleus of the target cell and, upon delivery, shRNA molecules are expressed and subsequently processed by intracellular enzymes into siRNA molecules that silence the expression of the mutant, disease-causing protein (Figure 2).

In the silence and replace approach (Figure 2):

- A DNA construct is delivered to the nucleus of the target cell by a gene therapy vector (A) such as an AAV vector;
- Once inside of the nucleus, the DNA construct drives the continuous production of shRNA molecules (B) which are processed by an enzyme called Dicer into siRNAs (C);
- The processed siRNA is incorporated into RISC and silences the target gene using the same mechanism shown in Figure 1; and
- When the DNA expression cassette is additionally comprised of a wild type transgene, upon entry of the DNA construct into the nucleus of the target cell via the use of the AAV vector, the DNA construct also drives the continuous production of wild type protein (to restore native intracellular biological processes).

Figure 2



Our strategy is to discover, develop and commercialize treatments that leverage the capabilities of ddRNAi and the silence and replace approach to disease management.

For selected product candidates, at the appropriate stage, we may collaborate with large biopharmaceutical companies to further co-develop and, if approved, commercialize our ddRNAi-based and silence and replace-based products to achieve broad clinical and commercial distribution. For specific clinical indications that we deem to be outside of our immediate areas of focus, we will continue to out-license, where appropriate, applications of our ddRNAi and silence and replace technology to facilitate the development of differentiated therapeutics, which could provide further validation of our proprietary technology and approach to disease management.

Our cash and cash equivalents will be deployed for the advancement of our product candidate BB-301 for the treatment of OPMD-derived dysphagia, including the natural history lead-in study and the Phase 1b/2a BB-301 treatment study, for the continued advancement of development activities for other existing and new product candidates, for general corporate purposes and for strategic growth opportunities.

Oculopharyngeal Muscular Dystrophy—OPMD

OPMD is an insidious, autosomal-dominant, late-onset degenerative muscle disorder that typically presents in patients at 40-to-50 years of age. The disease is characterized by progressive swallowing difficulties (dysphagia) and eyelid drooping (ptosis). OPMD is caused by a specific mutation in the poly(A)-binding protein nuclear 1, or PABPN1, gene. OPMD is a rare disease; however, patients have been diagnosed with OPMD in at least 33 countries. Patient populations suffering from OPMD are well-identified, and significant geographical clustering has been noted for patients with this disorder, which could simplify clinical development and global commercialization efforts.

BB-301 is an AAV-based gene therapy designed to silence the expression of disease-causing genes (to slow, or halt, the underlying mechanism of disease progression) and to simultaneously replace the mutant genes with normal, “wildtype” genes (to drive restoration of function in diseased cells). This fundamental therapeutic approach to disease management is called “silence and replace” and this biological mechanism offers the potential to restore the underlying physiology of the treated tissues and, in the process, improve treatment outcomes for patients suffering from the chronic and, potentially, fatal effects of Oculopharyngeal Muscular Dystrophy (OPMD). BB-301 has been granted Orphan Drug Designation in the United States and the European Union.

Our Strengths

We believe that the combination of our proprietary ddRNAi and silence and replace technology, and our deep expertise in the design and development of genetic medicines, will enable us to achieve and maintain a leading position in gene silencing and gene therapy for the treatment of human disease. Our key strengths include:

- A first mover advantage for silence and replace-based therapeutics;
- A proprietary ddRNAi-based silence and replace technology platform that may potentially enable the serial development of single-administration therapeutics capable of facilitating sustained, long-term silencing of disease-causing genes and concomitant replacement of wild type gene function;
- A proprietary AAV vector technology which improves the endosomal escape capability of virus produced in insect cells using a baculovirus system. This technology has broad application in AAV-based gene therapies;
- The capabilities to drive the development of a pipeline of programs focused on chronic diseases with either large patient populations, or rare diseases, which may potentially support the receipt of Orphan Drug Designation, including OPMD; and
- A growing portfolio of patents protecting improvements to our ddRNAi, and silence and replace, technology and product candidates through at least 2036, with additional patent life anticipated through at least 2040.

Our Strategy

We endeavor to become the leader in discovery, development, and commercialization of silence and replace-based therapeutic agents. We apply the following general strategy to drive the Company towards these goals:

- Selectively develop proprietary and partnered programs; and
- Continue to explore and secure research and development partnerships with global biopharmaceutical companies supported by the differentiated nature of our scientific platform and intellectual property portfolio.

Our senior leadership team will continue to explore partnership opportunities with global biopharmaceutical companies, as we expect that the unique attributes of the proprietary ddRNAi and silence and replace approaches, and the breadth of potential clinical indications amenable to our proprietary methods, to support the formation of collaborations over a broad range of diseases with significant unmet medical need.

We seek to actively protect our intellectual property and proprietary technology. These efforts are central to the growth of our business and include:

- Seeking and maintaining patents claiming our ddRNAi and silence and replace technologies and other inventions relating to our specific products in development or that are otherwise commercially and/or strategically important to the development of our business;
- Protecting and enforcing our intellectual property rights; and
- Strategically licensing intellectual property from third parties to advance development of our product candidates.

Our Pipeline

The following table sets forth our current product candidate and the development status:

Table 1. Pipeline: Oculopharyngeal Muscular Dystrophy

Benitec Pipeline Summary



Novel Technology Platform

- Benitec's DNA-directed RNA interference (ddRNAi) platform combines gene therapy with RNA interference (RNAi) to simultaneously silence & replace disease-causing genes permanently, following a single administration
- Platform has application in diseases that cannot be treated with gene silencing or gene therapy alone



Lead Asset Entered Clinical Evaluation in Orphan Disease in November 2023

- BB-301 is being developed to treat dysphagia (difficulty swallowing) in subjects with Oculopharyngeal Muscular Dystrophy (OPMD). There are no therapies approved for the treatment of OPMD. The estimated prevalence in the US, Europe, Canada & Israel is 15k subjects.
- Compelling preclinical data demonstrated complete restoration of muscle function in vivo via a murine disease model
- The Investigational New Drug (IND) application for BB-301 was approved to proceed by the FDA in June 2023
- The first study subject was safely treated in the BB-301 Phase 1b/2a clinical trial (NCT06185673) in November 2023. The second study subject was safely treated in February 2024. The third study subject was safely treated in October 2024. The fourth subject was safely treated in December 2024. The fifth study subject was safely treated in February 2025, and the sixth study subject was safely treated in April 2025.



Recent Milestones

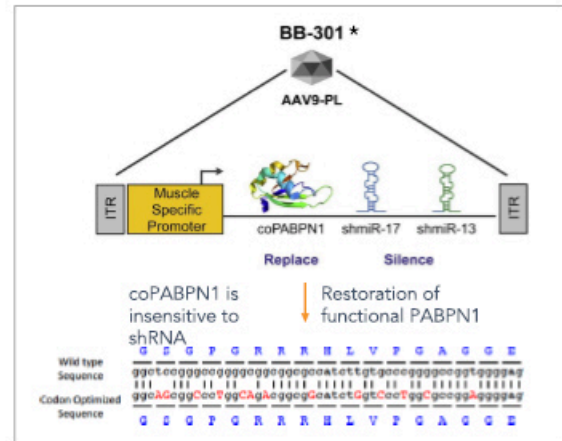
- Updated clinical safety data and clinical efficacy data for the BB-301 Phase 1b/2a clinical trial were disclosed in March 2025

We are developing BB-301 for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD)-related dysphagia. The Investigational New Drug (IND) application for BB-301 was approved to proceed by the U.S. Food and Drug Administration in June 2023. The first study subject was safely treated in the BB-301 Phase 1b/2a clinical trial (NCT06185673) in November 2023. The second study subject was safely treated in February 2024. The third study subject was safely treated in October 2024. The fourth study subject was safely treated in December 2024. The fifth study subject was safely treated in February 2025, and the sixth study subject was safely treated in April 2025. BB-301 is the lead investigational gene therapy agent under development by Benitec, and the key attributes of BB-301 are outlined in Figure 3.

BB-301 Simultaneously Silences Mutant PABPN1 & Delivers Wildtype PABPN1 To Restore Normal Myocyte Function

PABPN1 in OPMD

- PABPN1 is a ubiquitous protein that controls the length of mRNA poly(A) tails, mRNA export from the nucleus & alternative poly(A) site usage
- The PABPN1 mutant protein underlying OPMD is aggregation prone due to an N-terminal expanded poly-alanine tract of up to 18 contiguous alanine residues, and drives the formation of intranuclear inclusions (INIs) in the myocytes
- INIs also sequester wildtype PABPN1 and may contribute to the “loss of function” phenotype associated with OPMD



BB-301 is a first-in-class genetic medicine employing the “silence and replace” approach for the treatment of OPMD. OPMD is an insidious, autosomal-dominant, late-onset, degenerative muscle disorder that typically presents in patients at 40-to-50 years of age. The disease is characterized by progressive swallowing difficulties (dysphagia) and eyelid drooping (ptosis). OPMD is caused by a specific mutation in the poly(A)-binding protein nuclear 1 gene (PABPN1).

OPMD is a rare disease, however, patients have been diagnosed with OPMD in at least 33 countries. Patient populations suffering from OPMD are well-identified, and significant geographical clustering has been noted for patients with this disorder. Each of these attributes could facilitate efficient clinical development and global commercialization of BB-301.

PABPN1 is a ubiquitous factor that promotes the interaction between the poly(A) polymerase and CPSF (cleavage and polyadenylation specificity factor) and, thus, controls the length of mRNA poly(A) tails, mRNA export from the nucleus, and alternative poly(A) site usage. The characteristic genetic mutation underlying OPMD results in trinucleotide repeat expansion(s) within exon 1 of PABPN1 and results in an expanded poly-alanine tract at the N-terminal end of PABPN1. The mutation generates a protein with an N-terminal expanded poly-alanine tract of up to 18 contiguous alanine residues, and the mutant protein is prone to the formation of intranuclear aggregates designated as intranuclear inclusions (INIs). The INIs that sequester wildtype PABPN1 may contribute to the “loss of function” phenotype associated with OPMD.

No therapeutic agents are approved for the treatment of OPMD. Additionally, there are no surgical interventions available to OPMD patients that modify the natural history of the disease, which is principally comprised of chronic deterioration of swallowing function. BB-301 has received Orphan Drug Designation in the United States and the European Union and, upon achievement of regulatory approval for BB-301 in these respective jurisdictions, the Orphan Drug Designations would provide commercial exclusivity independent of intellectual property protection. While OPMD is a rare medical disorder, we believe the commercial opportunity for a safe and efficacious therapeutic agent in this clinical indication exceeds \$1 billion over the course of the commercial life of the product.

Investigational therapies that have been explored, unsuccessfully, in the past include:

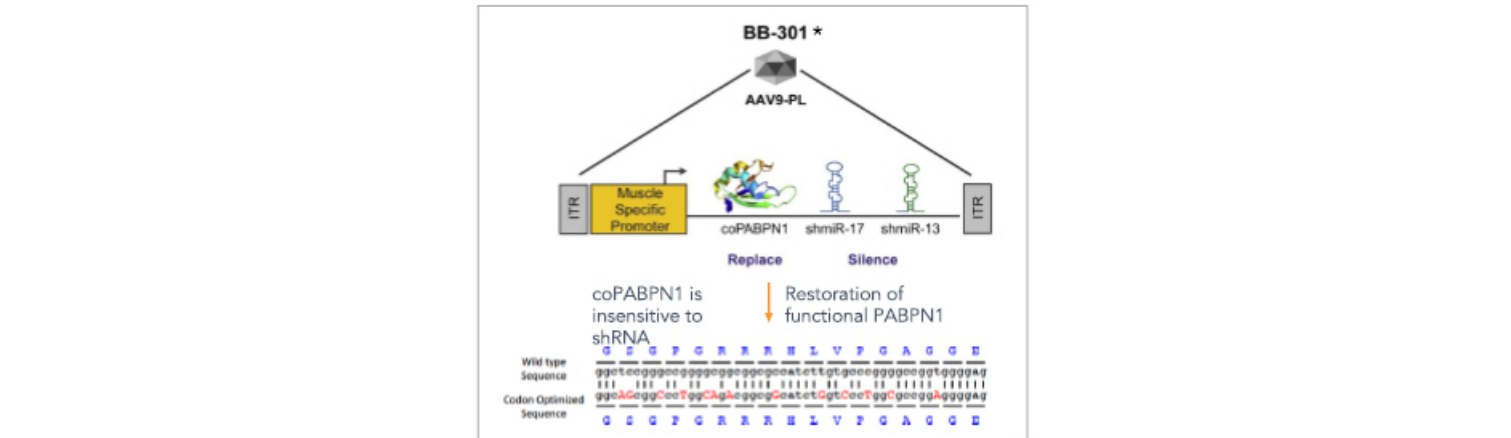
- Intravenous administration of trehalose; and
- The use of autologous myoblast transplant.

BB-301 is our Lead, Silence and Replace-Based, OPMD Therapeutic Agent

BB-301 is composed of a modified AAV serotype 9 (AAV9) capsid that expresses a bifunctional construct under the control of a single muscle specific Spc5-12 promoter to achieve co-expression of both the codon-optimized PABPN1 mRNA and two shmiR molecules directed against wild type and mutant PABPN1. BB-301 is designed to correct the genetic defect underlying OPMD following a single localized administration.

BB-301—Design and Mechanism of Action

BB-301 is designed to target two distinct regions of the PABPN1 mRNA to accomplish gene silencing via the concomitant expression of two distinct shmiRs from a single DNA construct (Figure 4). BB-301 is also engineered to drive the simultaneous expression of a codon-optimized, siRNA-resistant, version of the wild type PABPN1 gene (Figure 4).

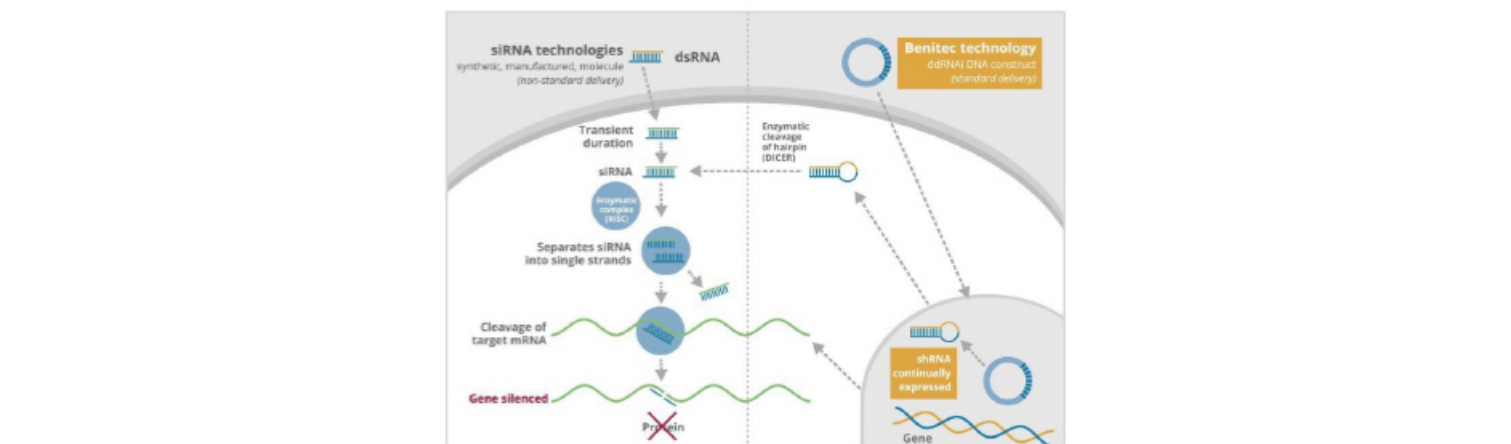


In collaboration with researchers at the Royal Holloway University of London and the Institut de Myologie in Paris, we developed a ddRNAi construct expressing three shRNAs against three distinct regions of PABPN1 mRNA and observed effective silencing of the PABPN1 gene in vitro using this ddRNAi construct. Furthermore, as part of this collaboration, we have generated a gene expression construct that produces a siRNA-resistant version of the wild type PABPN1 gene.

In subsequent studies undertaken exclusively by Benitec, a second set of target regions within PABPN1 were identified for therapeutic development and shmiRs designed against these regions. Additional shmiRs have also been designed for the original shRNA developed in collaboration with Royal Holloway University of London and the Institut de Myologie. The ‘silence and replace’ construct, designated BB-301, incorporates the two best performing shmiRs, and the gene expression construct that produces a siRNA-resistant version of the wild type PABPN1 gene, under the control of a muscle-specific promoter. The mechanism of action of BB-301 is shown in Figure 5.

Figure 5

ddRNAi Mechanism of Action



In initial in vivo studies evaluating the use of direct intramuscular injection of AAV-based constructs with the potential to facilitate the desired silence and replace approach in the A17 transgenic mouse model of OPMD at the Royal Holloway University of London and the Institut de Myologie, we observed decreases in muscle fibrosis, increases in cross sectional area of the treated muscles, decreases in intranuclear inclusions, and normalization of muscle strength. These nonclinical results were published in Nature Communications in April 2017.

In subsequent studies, Benitec demonstrated in a key nonclinical model (the A17 mouse model) that a single intramuscular injection of BB-301 results in robust intracellular silencing of PABPN1 protein production and concomitant expression of the normal, biologically functional PABPN1 protein. In the A17 mouse model, the treatment restores muscle strength and muscle weight to wild type levels and improves other physiological hallmarks of the disease (Figure 6a, Figure 6b, Figure 6c, Figure 6d):

- Multiple A17 animal cohorts received single doses of BB-301 (over a range of doses spanning 4×10^8 vg/muscle-to- 7.5×10^{11} vg/muscle) and, following BB-301 administration, each cohort was observed for 14-weeks
- BB-301 was injected into the Tibialis Anterior (TA) muscle of 10 week old-to-12 week old animals and, 14-weeks post administration, each A17 cohort was anesthetized and the contractile properties of the injected TA muscles were analyzed via in-situ muscle electrophysiology
- Intermediate doses of BB-301 resulted in 75% silencing of PABPN1 and 26% replacement of wild type PABPN1 activity, leading to full restoration of muscle strength, clearance of INIs, and a reduction of fibrosis
- An additional experiment conducted over the course of 20-weeks demonstrated that more modest doses of BB-301 (which supported only partial resolution of the disease phenotype at week-14) were, surprisingly, able to facilitate significant benefit at 20-weeks, as evidenced by restoration of parameters relating to muscle strength, weight and INI formation

Figure 6a. Dose-Dependent shRNA Expression

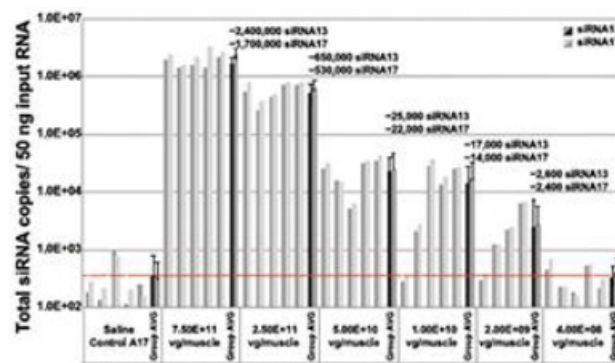


Figure 6b. Dose-Dependent PABPN1 Inhibition and Transgene Expression

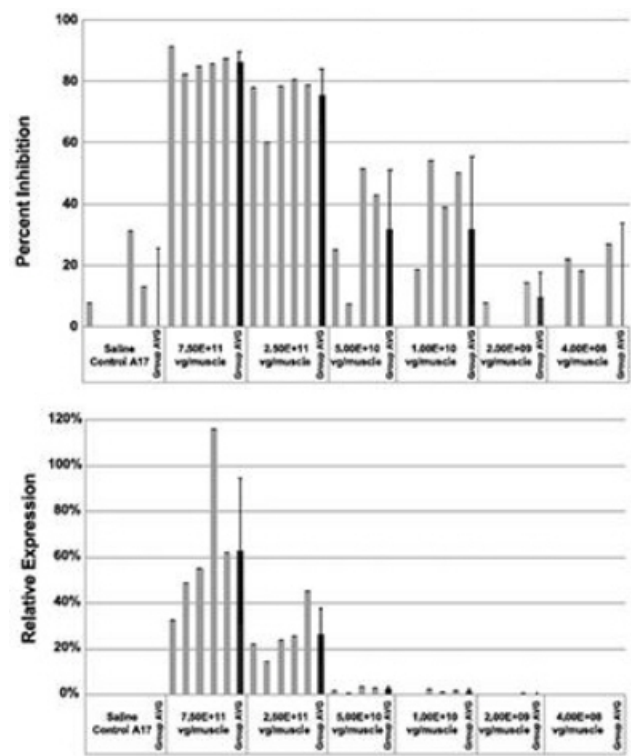


Figure 6c. Dose-Dependent Decreases in Intracellular Inclusions

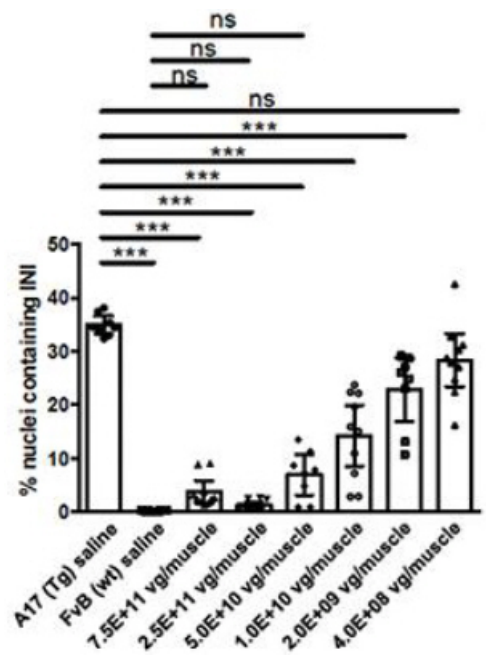
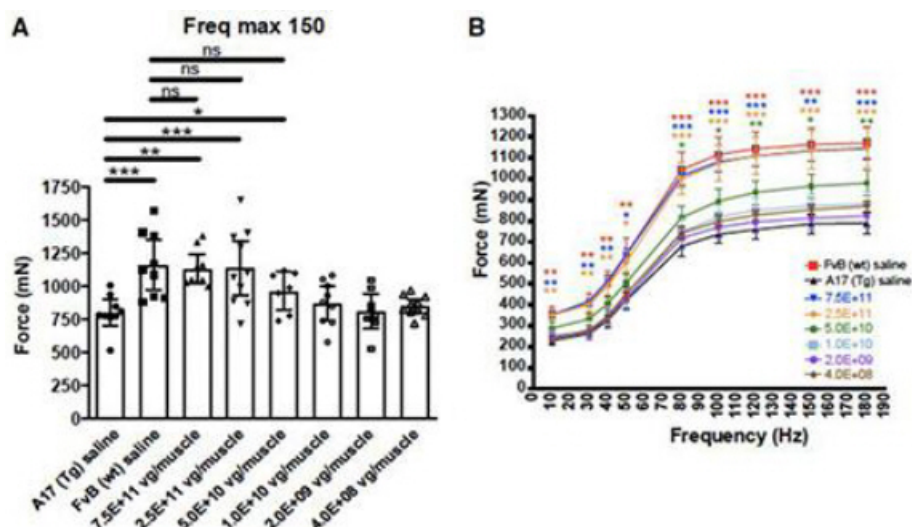


Figure 6d. Dose-Dependent Increases in Muscle Force



Restoration of muscle strength was assessed by muscle contractility measurements in response to a series of induced impulses that ranged from 10 to 180 Hz

Ongoing Development Activities for BB-301

On July 8, 2020, Benitec announced the initiation of the BB-301 Pilot Dosing Study in large animal subjects.

The BB-301 Pilot Dosing Study was the first of two planned CTA-enabling and IND-enabling studies that were designed to be conducted in large animals. The BB-301 Pilot Dosing Study was carried out under the guidance of the scientific team at Benitec, with key elements of the study design and execution conducted in close collaboration with a team of leading experts in both medicine and surgery that have been deeply engaged in the treatment of OPMD patients for several decades. The BB-301 Pilot Dosing Study, along with the subsequent GLP Toxicology and Biodistribution Study, were conducted in canine subjects and were carried out to support the validation and optimization of the newly designed method of BB-301 administration, confirm the efficiency of vector transduction and transgene expression in the key tissue compartments underlying the natural history of OPMD, confirm the optimal drug doses in advance of initiation of human clinical studies, and facilitate observation of key toxicological data-points.

The BB-301 Pilot Dosing Study was designed as an 8-week study in Beagle dogs to confirm the transduction efficiency of BB-301 upon administration via direct intramuscular injection into specific anatomical regions of the pharynx through the use of an open surgical procedure. This new route of BB-301 administration was developed in collaboration with key surgical experts in the field of Otolaryngology, and this novel method of BB-301 dosing was implemented to significantly enhance the ability of a treating physician to accurately administer the AAV-based investigational agent to the muscles that underlie the characteristic deficits associated with the progression of OPMD. It is important to note that prior nonclinical studies of BB-301 have reproducibly validated the robust biological activity achieved following direct intramuscular injection. As an example, direct injection of BB-301 into the tibialis anterior muscles of A17 mice facilitated robust transduction of the targeted skeletal muscle cells and supported complete remission of the OPMD disease phenotype in this animal model.

Benitec conducted the BB-301 Pilot Dosing Study in Beagle dog subjects to demonstrate that direct intramuscular injection of BB-301 via the use of a proprietary dosing device in an open surgical procedure could safely achieve the following goals:

- Biologically significant and dose-dependent levels of BB-301 tissue transduction (i.e., delivery of the multi-functional BB-301 genetic construct into the target pharyngeal muscle cells);
- Broad-based and dose-dependent expression of the three distinct genes comprising the BB-301 gene construct within the pharyngeal muscle cells; and
- Durable and biologically significant levels of target gene knock-down (i.e., inhibition of the expression of the gene of interest) within the pharyngeal muscle cells.

The Pilot Dosing Study evaluated the safety and biological activity of two concentrations of BB-301 (1.0+E13 vg/mL and 3.0+E13 vg/mL) across three distinct doses (1.0+E13 vg/mL, 3.0+E13 vg/mL with a low injection volume, and 3.0+E13 vg/mL with a high injection volume) following direct intramuscular injection into the Hypopharyngeal (HP) muscles and the

Thyropharyngeal (TP) muscles of Beagle dogs via the use of a proprietary delivery device employed in an open surgical procedure. The HP muscle in Beagle dogs corresponds to the Middle Pharyngeal Constrictor muscle in human subjects, and the TP muscle in Beagle dogs corresponds to the Inferior Pharyngeal Constrictor muscle in human subjects. Atrophy, fibrosis, and the presence of intranuclear inclusions characterize the Middle Pharyngeal Constrictor muscles and the Inferior Pharyngeal Constrictor muscles of human subjects diagnosed with OPMD. BB-301 was injected into the pharyngeal muscles of the Beagle dog subjects only on Day 1 of the Pilot Dosing Study, and the corresponding canine pharyngeal muscles were harvested for analysis after 8 weeks of observation post-dosing. BB-301 dosing was carried out independently by both a veterinary surgeon and a practicing Otolaryngologist who has extensive experience with the provision of palliative surgical care for OPMD patients.

The key results are summarized here:

Figure 7. Pharyngeal Muscle Tissue Transduction Levels for BB-301

BB-301 Dose (vg/mL)	Copies of BB-301 (average copies per cell)	
	Hypopharyngeal Muscle	Thyropharyngeal Muscle
3.00 x 10 ¹³ vg/ml <i>High Volume</i>	5.12	5.66
3.00 x 10 ¹³ vg/ml <i>Low Volume</i>	3.15	2.70
1.00 x 10 ¹³ vg/ml	1.52	2.06

Regarding Gene Expression Levels Observed for BB-301 Within the Pharyngeal Muscle Tissues (Figure 8, Figure 9):

- BB-301 encodes two distinct siRNA species (i.e., siRNA13 and siRNA17) which are each, independently, capable of inhibiting (i.e., “silencing”) the expression of the mutant form of the PABPN1 protein and the wild type (i.e., endogenous) form of the PABPN1 protein (importantly, the mutant form of the PABPN1 protein underlies the development and progression of OPMD).
- BB-301 also codes for a wild type version of the PABPN1 protein whose intracellular expression is unaffected by the inhibitory activities of siRNA13 and siRNA17, and this codon optimized PABPN1 protein (i.e., coPABPN1) serves to replenish the endogenous form of the PABPN1 protein and to replace the mutant form of PABPN1 that underlies the development and progression of OPMD in diseased tissues.
- For comparative purposes, it should be noted that the average range of expression for wild type PABPN1 within the pharyngeal muscle cells of Beagle dogs is 4.5 copies per cell-to-7.8 copies per cell.

Figure 8. siRNA13 Expression Levels for BB-301 within Pharyngeal Muscle Tissues and siRNA17 Expression Levels for BB-301 within Pharyngeal Muscle Tissues

BB-301 Dose (vg/mL)	siRNA13		siRNA17	
	Hypopharyngeal Muscle	Thyropharyngeal Muscle	Hypopharyngeal Muscle	Thyropharyngeal Muscle
	average copies per cell	average copies per cell	average copies per cell	average copies per cell
3.00 x 10 ¹³ vg/ml <i>High Volume</i>	340,613	518,329	64,393	112,783
3.00 x 10 ¹³ vg/ml <i>Low Volume</i>	221,663	303,516	41,787	59,723
1.00 x 10 ¹³ vg/ml	83,168	136,812	17,321	30,253

Figure 9. coPABPN1 Expression Levels for BB-301 within Pharyngeal Muscle Tissues

BB-301 Dose (vg/mL)	Copies of coPABPN1 (average copies per cell)	
	Hypopharyngeal Muscle	Thyropharyngeal Muscle
3.00 x 10 ¹³ vg/ml <i>High Volume</i>	61.69	77.26
3.00 x 10 ¹³ vg/ml <i>Low Volume</i>	27.43	62.89
1.00 x 10 ¹³ vg/ml	17.54	30.84

Regarding Wild Type PABPN1 Silencing (i.e., target “knock-down”) Observed for BB-301 Within the Pharyngeal Muscle Tissues (Figure 10):

- As noted above, BB-301 encodes two distinct siRNA species (i.e., siRNA13 and siRNA17) which are each, independently, capable of inhibiting (i.e., “silencing”) the expression of all forms of the PABPN1 protein (siRNA13 and siRNA17 silence the expression of both wild type PABPN1 [wtPABPN1] and mutant PABPN1).
- While the Beagle dog subjects treated in the BB-301 Pilot Dosing Study did not express mutant PABPN1, the level of BB-301-driven gene silencing for the PABPN1 target can be accurately assessed due to the equivalent inhibitory effects of siRNA13 and siRNA17 on both wtPABPN1 and mutant PABPN1.
- Thus, the wtPABPN1 silencing activity observed in the BB-301 Pilot Dosing Study served as a surrogate for the activity that would be anticipated in the presence of mutant PABPN1.
- BB-301 has been evaluated in prior nonclinical studies in animals that express mutant PABPN1 and manifest the key signs and symptoms of OPMD and, in these animal models of OPMD, the achievement of PABPN1 silencing levels of 31% inhibition or higher led to resolution of OPMD disease symptoms and correction of the histological hallmarks of OPMD.

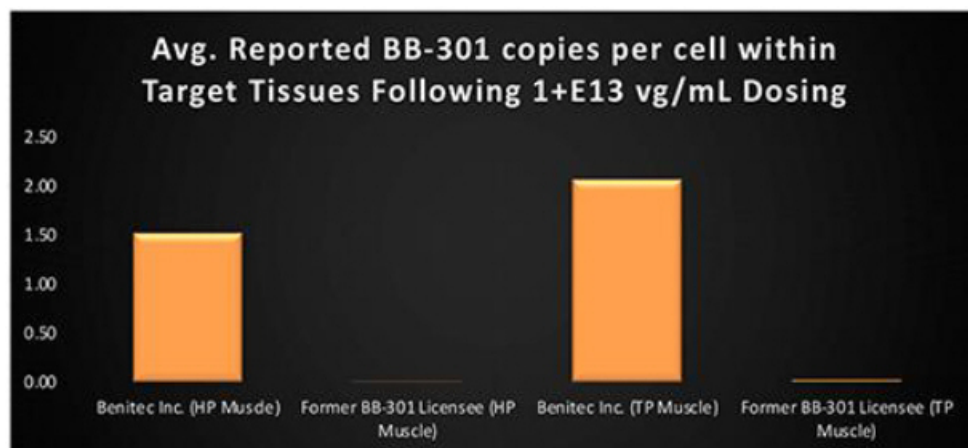
Figure 10. PABPN1 Silencing (i.e., “target knock-down”) within Pharyngeal Muscle Tissues

BB-301 Dose (vg/mL)	Average Reported % Inhibition of wtPABPN1	
	Hypopharyngeal Muscle	Thyropharyngeal Muscle
3.00 x 10 ¹³ vg/ml <i>High Volume</i>	83%	82%
3.00 x 10 ¹³ vg/ml <i>Low Volume</i>	74%	64%
1.00 x 10 ¹³ vg/ml	60%	69%

Finally, it is critical to highlight the key methodological distinctions between the BB-301 Pilot Dosing Study in Beagle dogs conducted by Benitec (i.e., the study described above) and the prior Beagle dog dosing study carried out independently by the previous BB-301 licensee. The BB-301 dosing study conducted by the prior BB-301 licensee employed non-ideal routes and methods of BB-301 administration to the target pharyngeal muscle tissues and employed similarly limited analytical methods at the completion of the dosing phase of the study. The Benitec team worked to optimize the route and method of administration of BB-301 and to refine the core analytical methods employed following the completion of dosing.

Following the implementation of these methodological modifications, Benitec demonstrated a 248-fold improvement (+24,650%) in BB-301 transduction of the HP muscle and a 111-fold improvement (+11,027%) in BB-301 transduction of the TP muscle relative to the levels of BB-301 transduction observed by the previous BB-301 licensee (Figure 11).

Figure 11. Impact of Benitec-Initiated Methodological Improvements on the Relative Pharyngeal Muscle Tissue Transduction Levels Achieved for BB-301



Following the disclosure of the positive interim BB-301 Pilot Dosing Study results, Benitec completed pre-CTA and pre-IND meetings with regulatory agencies in France, Canada, and the United States.

Summary of the Key Regulatory Interactions:

- In June 2023 the U.S. Food and Drug Administration (FDA) cleared the Investigational New Drug (IND) application for BB-301 which allowed dosing of BB-301 to begin for OPMD subjects that are eligible for enrollment into the Phase 1b/2a treatment study (NCT06185673) described below.

Operational Updates

The key milestones related to the development of BB-301 for the treatment of OPMD, along with other corporate updates, are outlined below:

BB-301 Clinical Development Program Overview:

- The BB-301 clinical development program is being conducted in the United States, and the primary elements of the program are summarized below:
 - The program comprises approximately 76 weeks of follow-up which will consist of:
 - The OPMD Natural History (NH) Study: 6-month pre-treatment observation periods for the evaluation of baseline disposition and natural history of OPMD-derived dysphagia (swallowing impairment) in each study participant.
 - Dosing with BB-301: 1-day of BB-301 dosing to initiate participation in the Phase 1b/2a single-arm, open-label, sequential, dose-escalation cohort study (NCT06185673). BB-301 is delivered directly to the pharyngeal muscles of each study subject.
 - Phase 1b/2a Treatment Evaluation: 52-weeks of post-dosing follow-up for conclusive evaluation of the primary and secondary endpoints of the BB-301 Phase 1b/2a treatment study (NCT06185673), with interim safety and efficacy results expected to be available at the end of each 180-day period following the administration of BB-301.

- The OPMD NH Study will characterize the level of dysphagia borne by each OPMD subject at baseline and assess subsequent progression of dysphagia via the use of the following quantitative radiographic measures (i.e., videofluoroscopic swallowing studies or “VFSS”). The VFSS outlined below collectively provide objective assessments of global swallowing function and the function of the pharyngeal constrictor muscles (i.e., the muscles whose functional deterioration drives disease progression in OPMD):
 - Total Pharyngeal Residue $\%(C2-4)^2$
 - Pharyngeal Area at Maximum Constriction (PhAMPC)
 - Dynamic Imaging Grade of Swallowing Toxicity Scale (DIGEST)
 - Vallecular Residue $\%(C2-4)^2$, Pyriform Sinus Residue $\%(C2-4)^2$, and Other Pharyngeal Residue $\%(C2-4)^2$
 - Normalized Residue Ratio Scale (NRRS_v, NRRS_p)
 - Pharyngeal Construction Ratio (PCR)
- The NH study will also employ clinical measures of global swallowing capacity and oral-pharyngeal dysphagia, along with two distinct patient-reported outcome instruments targeting the assessment of oral-pharyngeal dysphagia.
- Upon the achievement of 6-months of follow-up in the NH Study, participants will, potentially, be eligible for enrollment into the BB-301 Phase 1b/2a treatment study (NCT06185673).
- BB-301 Phase 1b/2a Treatment Study (NCT06185673):
 - This first-in-human (FIH) study will evaluate the safety and clinical activity of intramuscular doses of BB-301 administered to subjects with OPMD.
 - The primary endpoint of the FIH study will be safety.
 - Secondary endpoints are designed to determine the impact of BB-301 on swallowing efficiency, swallowing safety, and pharyngeal constrictor muscle function in subjects diagnosed with OPMD with dysphagia via the use of serial clinical and videofluoroscopic assessments. Critically, each of the clinical and videofluoroscopic assessments employed in the FIH study will be equivalent to those employed for the NH study to facilitate comparative clinical and statistical analyses for each study subject.
 - The primary and secondary endpoints will be evaluated during each 90-day period following BB-301 intramuscular injection (Day 1).
 - The NH of dysphagia observed for each OPMD NH Study participant, as characterized by the VFSS and clinical swallowing assessments carried out during the NH Study, will serve as the baseline for comparative assessments of safety and efficacy of BB-301 upon rollover from the NH Study onto the BB-301 Phase 1b/2a Treatment Study (NCT06185673).
- In December 2022, Benitec began screening OPMD subjects for the NH Study at the lead clinical study site in the United States.
- In January 2023, Benitec announced the enrollment of the first OPMD subject into the NH Study in the United States.
- In November 2023, Benitec announced the completion of the safe administration of BB-301 to the first study subject in the Phase 1b/2a clinical study (NCT06185673) in the United States. The second study subject was safely treated with BB-301 in February 2024, the third study subject was safely treated in October 2024. The fourth study subject was safely treated in December 2024. The fifth study subject was safely treated in February 2025, and the sixth study subject was safely treated in April 2025.
- As of January 2024, 23 subjects had enrolled into the NH study in the United States.

On March 19, 2025 Benitec reported positive interim clinical trial data for the first three subjects enrolled into the BB-301 Phase 1b/2a Treatment Study (NCT06185673).

Subjects Enrolled into the BB-301 Clinical Development Program are Impacted by Two Discrete Drivers of Total Dysphagic Symptom Burden:

- OPMD Subjects enrolled into the OPMD Natural History Study and the BB-301 Phase 1b/2a Clinical Treatment Study can be impacted by the post swallow accumulation of food and liquid (“Inefficient Swallowing”).
- OPMD Subjects enrolled into the OPMD Natural History Study and the BB-301 Phase 1b/2a Clinical Treatment Study can be impacted by pathologic sequential swallows comprising rapid involuntary contractions of the pharyngeal muscles without restoration of the resting pharyngeal diameter between pharyngeal contractions (“Ineffective Swallowing”).

Summary of the Interim Clinical Study Results for Subject 1, Subject 2, and Subject 3:

- Three Subjects with distinct causes of their respective dysphagic symptom burdens were safely treated with BB-301 (1.2e13 vg/Subject) and experienced significant, clinically meaningful improvements in swallowing function.
- There were no Severe Adverse Events.
- All three Subjects experienced significant reductions in their total dysphagic symptom burdens:
 - Subject 1, plagued by Inefficient Swallowing, experienced clinically significant reductions in post swallow accumulation of foods and liquids per the VFSS Total Pharyngeal Residue (TPR) results and achieved a correspondingly significant reduction in total dysphagic symptom burden per the Total Sydney Swallow Questionnaire (SSQ) Scores 12-months post-BB-301 administration. This Subject has completed the statistical follow-up period of the BB-301 Phase 1b/2a Treatment Study.
 - Subject 2, plagued by Ineffective Swallowing, experienced an almost complete resolution of pathologic sequential swallows per the VFSS results and achieved a correspondingly significant reduction in total dysphagic symptom burden per the Total SSQ Scores, achieving an SSQ score indicative of a clinically normal swallowing profile 12-months post-BB-301 administration. This Subject has completed the statistical follow-up period of the BB-301 Phase 1b/2a Treatment Study.
 - Subject 3, plagued by Ineffective Swallowing, experienced complete resolution of pathologic sequential swallows per the VFSS results and achieved a correspondingly significant reduction in total dysphagic symptom burden per the Total SSQ Score, achieving an SSQ score indicative of a clinically normal swallowing profile 3-months post BB-301 administration.

Clinical Study Results for Subject 1 (365-Days Post Treatment with BB-301):

Subject 1, plagued by Inefficient Swallowing, experienced significant, clinically meaningful reductions of post swallow residue across all food and liquid consistencies 12-months post treatment with BB-301 per the VFSS results, and the VFSS results were accompanied by significant reductions in total dysphagic symptom burden.

Subject 1 displayed significant reductions (i.e., improvements) in VFSS TPR (37% reduction for Thin Liquid, 18% reduction for Solid Food, and 29% reduction for Thick Liquids) following the administration of the low-dose of BB-301 as compared to the average values recorded for Subject 1 during the pre-treatment period.

Subject 1 also displayed continued clinically meaningful reductions (i.e., improvements) in total dysphagic symptom burden with an average 12-month post-treatment SSQ Total Score demonstrating a 41% reduction as compared to the average values recorded for Subject 1 during the pre-treatment period.

Clinical Study Results for Subject 2 (365-Days Post Treatment with BB-301):

Subject 2, plagued by Ineffective Swallowing, experienced significant, clinically meaningful reductions in the frequency of pathologic sequential swallows 12-months post treatment with BB-301 per the VFSS results, and the VFSS results were accompanied by significant reductions in total dysphagic symptom burden with Subject 2 achieving an SSQ score indicative of a clinically normal swallowing profile.

During the fifteen pre-treatment VFSS assessments conducted for Thin Liquid in the OPMD Natural History Study, Subject 2 experienced a high frequency of pathologic sequential swallows (observed during 80% of the swallowing assessments). During the twelve post-treatment VFSS assessments conducted for Thin Liquid in the Phase 1b/2a Clinical Treatment Study, Subject 2 experienced a significantly lower frequency of pathologic sequential swallows (observed during 17% of the swallowing assessments). Critically, the magnitude of reduction in the frequency of pathologic sequential swallows reported for Thin Liquid at the 6-month post-treatment interim clinical update in October 2024 (observed during 17% of the swallowing assessments) was maintained at month 12 (again observed during 17% of the swallowing assessments).

Subject 2 also displayed continued clinically meaningful reductions (i.e., improvements) in total dysphagic symptom burden with an average 12-month post-treatment SSQ Total Score demonstrating a 91% reduction as compared to the average values recorded for Subject 2 during the pre-treatment period. The 12-month post-treatment average SSQ value of 68 units for Subject 2 represents a clinically normal swallowing profile.

Interim Clinical Study Results for Subject 3 (90-Days Post Treatment with BB-301):

Subject 3, plagued by Ineffective Swallowing, experienced significant, clinically meaningful reductions in the frequency of pathologic sequential swallows 3-months post treatment with BB-301 per the VFSS results, and the VFSS results were accompanied by a significant reduction in total dysphagic symptom burden with Subject 3 achieving an SSQ score indicative of a clinically normal swallowing profile.

During the twenty-five pre-treatment VFSS assessments conducted for Thin Liquid and Thick Liquids in the OPMD Natural History Study, Subject 3 experienced a high frequency of pathologic sequential swallows (observed during 84% of the swallowing assessments). During the five post-treatment VFSS assessment conducted for Thin Liquid and Thick Liquids in the Phase 1b/2a Clinical Treatment Study, Subject 3 experienced no pathologic sequential swallows (observed during 0% of the swallowing assessments).

Subject 3 also displayed a clinically meaningful reduction (i.e., improvement) in total dysphagic symptom burden with a 3-month post-treatment SSQ Total Score demonstrating a 68% reduction as compared to the average values recorded for Subject 3 during the pre-treatment period. The 3-month post-treatment SSQ value of 70 units for Subject 3 represents a clinically normal swallowing profile.

The Subjects were blinded to their SSQ Total Scores and VFSS (TPR and pathologic sequential swallowing frequency) assessment results, and the Central Reader for the VFSS assessments was blinded to the SSQ Total Scores for each Subject.

Adverse Events:

No Severe Adverse Events have been observed for the Subjects treated with BB-301.

As of May 2025, six subjects have been safely treated with BB-301.

Manufacturing

The manufacture of the biological products required for gene therapy is complex and difficult. We do not currently own or operate manufacturing facilities for the production of preclinical, clinical or commercial quantities of any of our product candidates. We are exploring long-term manufacturing alliances with a number of potential partners to investigate manufacturing processes in order to produce materials at reasonable scale and cost of goods to support future commercialization efforts. We do not have a long-term agreement with any third-party manufacturer, but we plan to establish such a relationship with an appropriate manufacturer to serve our long-term needs.

Manufacturing is subject to extensive regulations that impose various procedural and documentation requirements, which govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance, among others. Our contract manufacturing organizations manufacture our product candidates under cGMP conditions. cGMP is a regulatory standard for the production of pharmaceuticals that will be used in humans.

Sales and Marketing

We have not yet established sales, marketing or product distribution operations because our product candidates are in preclinical or clinical development. If we receive marketing and commercialization approval for any of our product candidates, we intend to market the product through strategic alliances and distribution agreements with third parties. In certain cases, we may market an approved product directly worldwide or in selected geographical segments. The ultimate implementation of our strategy for realizing the financial value of our product candidates is dependent on the results of clinical trials for our product candidates, the availability of funds and the ability to negotiate acceptable commercial terms with third parties.

Competition

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies.

Any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our proprietary technology and scientific expertise in

gene silencing using ddRNAi provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions and governmental agencies and public and private research institutions that may develop potentially competitive products or technologies. We are aware of several companies focused on developing gene therapy or gene silencing product candidates.

We are not aware of any companies developing a gene therapy or gene silencing approach for OPMD. Our product candidates, if approved, would also compete with treatments that have already been approved and accepted by the medical community, patients and third-party payers.

Many of our competitors and potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical study sites and subject registration for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new products enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of competition and the availability of reimbursement from government and other third party-payers.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, we expect that our therapeutic products, if approved, will be priced at a significant premium over competitive products and our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of competitive products including biosimilar or generic products.

This increasingly competitive landscape may compromise the development of our product candidates.

Royalties, milestone payments and other license fees

We are required to pay royalties, milestone payments and other license fees in connection with our licensing of intellectual property from third parties, including as discussed below.

Foreign Currency Translation and Other Comprehensive Income (Loss)

The Company's functional currency and reporting currency is the United States dollar. BBL's functional currency is the Australian dollar (AUD). Assets and liabilities are translated at the exchange rate in effect at the balance sheet date. Revenues and expenses are translated at the average rate of exchange prevailing during the reporting period. Equity transactions are translated at each historical transaction date spot rate. Translation adjustments arising from the use of different exchange rates from period to period are included as a component of stockholders' equity as "Accumulated other comprehensive loss." Gains and losses resulting from foreign currency translation are included in the consolidated statements of operations and comprehensive loss as other comprehensive income (loss).

ATM Agreement

On October 11, 2024, the Company entered into a Sales Agreement (the “Sales Agreement”) with Leerink Partners LLC (the “Agent”). Pursuant to the terms of the Sales Agreement, the Company may offer and sell shares of the Company’s common stock having an aggregate offering amount of up to \$75 million from time to time through the Agent. The Agent will use its commercially reasonable efforts, as the agent and subject to the terms of the Sales Agreement, to sell the shares offered. Sales of the shares, if any, may be made in sales deemed to be an “at-the-market offering” as defined in Rule 415 under the Securities Act of 1933, as amended. The Company may also agree to sell shares to the Agent as principal for its own account on terms agreed to by the Company and the Agent. The Agent will be entitled to a commission from the Company of 3.0% of the gross proceeds from the sale of shares sold under the Sales Agreement. In addition, the Company has agreed to reimburse certain expenses incurred by the Agent in connection with the offering. Shares sold pursuant to the Sales Agreement, if any, will be sold pursuant to the Company’s shelf registration statement on Form S-3 (File No. 333-277310), that was filed with the Securities and Exchange Commission, including the related prospectus, dated March 5, 2024, as supplemented by a prospectus supplement.

March 25, 2025 Capital Raise

On March 25, 2025, the Company entered into an Underwriting Agreement with Leerink Partners LLC and TD Securities (USA) LLC, as representatives of the several underwriters named therein, pursuant to which the Company agreed to issue and sell, in an underwritten offering by the Company (the “Underwritten Offering”), (i) 1,143,000 shares of the Company’s common stock, par value \$0.0001 per share (the “Common Stock”) at a purchase price to investors of \$13.00 per share, and (ii) pre-funded warrants to purchase 300,000 shares of Common Stock at an exercise price of \$0.0001 per share at a purchase price to investors of \$12.999 per warrant, and a Securities Purchase Agreement with Averill Master Fund, Ltd. and Averill Madison Master Fund, Ltd. (together, the “Purchasers”), pursuant to which the Company agreed to issue and sell to the Purchasers an aggregate of 900,000 shares of Common Stock at a purchase price of \$13.00 per share in a registered direct offering (the “Direct Offering,” and together with the Underwritten Offering, the “Offerings”), the same price per share as the offering price in the Underwritten Offering. The Company received gross proceeds of approximately \$30.5 million and net proceeds of approximately \$28.2 million from the Offerings.

Results of Operations

Revenues

The Company has not generated any revenues from the sales of products. Revenues from licensing fees are included in the revenue from customers line item on our consolidated statements of operations and comprehensive loss. Our licensing fees have been generated through the licensing of our ddRNAi technology to biopharmaceutical companies. The Company did not recognize any revenue during the three and nine months ended March 31, 2025 and March 31, 2024.

Royalties and License Fees

Royalties and license fees consist primarily of payments we are required to remit for royalties and other payments related to in-licensed intellectual property. Under our in-license agreements, we may pay up-front fees and milestone payments and be subject to future royalties. We cannot precisely predict the amount, if any, of royalties we will owe in the future, and if our calculations of royalty payments are incorrect, we may owe additional royalties, which could negatively affect our results of operations. As our product sales increase, we may, from time to time, disagree with our third-party collaborators as to the appropriate royalties owed, and the resolution of such disputes may be costly, may consume management's time, and may damage our relationship with our collaborators. Furthermore, we may enter into additional license agreements in the future, which may also include royalty, milestone, and other payments.

Research and Development Expenses

Research and development expenses relate primarily to the cost of conducting clinical and preclinical trials. Preclinical and clinical development costs are a significant component of research and development expenses. The Company records accrued liabilities for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and includes these costs in trade and other payables on the consolidated balance sheets and within research and development expenses on the consolidated statements of operations and comprehensive loss.

The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance at the end of each reporting period. As actual costs become known, the Company adjusts its accrued liabilities. The Company has not experienced any material differences between accrued costs and actual costs incurred.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, related benefits, travel, and equity-based compensation expense. General and administrative expenses also include facility expenses, professional fees for legal, consulting, accounting and audit services and other related costs.

We anticipate that our general and administrative expenses may increase as the Company focuses on the continued development of the clinical OPMD program. The Company also anticipates an increase in expenses relating to accounting, legal and regulatory-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums and other similar costs.

Ms. Boston was appointed as the Company's Chief Financial Officer effective January 1, 2025. On December 9, 2024, the Compensation Committee approved increases of the base salaries of Dr. Jerel Banks and Megan Boston to \$667,000 and \$415,000, respectively, each adjustment being effective as of January 1, 2025. The Compensation Committee further determined that the target annual discretionary bonus with respect to the Company's 2025 fiscal year for Dr. Jerel Banks and Megan Boston will be 55% and 40% of their base salary, respectively. On December 9, 2024, the Board of Directors appointed Sophie Mukadam as Chief Operating Officer of the Company, effective as of January 1, 2025. Sophie Mukadam will receive a base salary of \$500,000 and a target annual bonus of 40% of base salary. On February 13, 2025, the Compensation Committee of the Company's board of directors approved a change to the base salary of Megan Boston, the Company's CFO, from USD\$415,000 to USD\$531,900 to be effective March 1, 2025. The base salary change was approved in anticipation of Ms. Boston relocating from Australia to Los Angeles, California. In connection with such relocation, the Compensation Committee also approved moving and transitional housing allowances in aggregate amounts of approximately \$5,000 and \$18,000, respectively.

Operating Expenses

The following tables sets forth a summary of our expenses for each of the periods:

	Three Months Ended March 31,		Nine Months Ended March 31,	
	2025	2024	2025	2024
	(US\$'000)			
Operating Expenses:				
Royalties and License Fees	\$ —	\$ (3)	\$ —	\$ (108)
Research and development	5,980	2,566	14,637	12,097
General and administrative	4,208	1,578	9,952	4,953
Total operating expenses	\$10,188	\$4,141	\$24,589	\$16,942

During the three and nine months ended March 31, 2025 and March 31, 2024, we incurred royalties and license fees expenses of zero and \$(3) thousand and zero and \$(108) thousand, respectively. The credits to expense during the three and nine months ended March 31, 2024 relate to the reversal of accruals for license fees no longer due.

During the three and nine months ended March 31, 2025, respectively, we incurred \$6.0 million and \$14.6 million in research and development expenses, respectively, as compared to \$2.6 million and \$12.1 million for the comparable periods ended March 31, 2024. Research and development expenses relate primarily to ongoing clinical development of BB-301 for the treatment of OPMD. The year-over-year increase for the three and nine months ended March 31, 2025 reflects the timing of contract manufacturing activity and the timing of payments for the OPMD Natural History and Dosing study.

General and administrative expense totaled \$4.2 million and \$10.0 million for the three and nine months ended March 31, 2025, compared to \$1.6 million and \$5.0 million for the comparable periods ended March 31, 2024. The increase for the three month period, 2025 relates primarily to an increase in share based compensation of \$2.1 million, travel expenses of \$176 thousand and salaries and wages of \$254 thousand. The increase in the nine month period relates to higher corporate costs related to an increase in legal fees, and higher travel expenses as well as an increase in salaries and wages.

Other Income (Expense)

The following tables sets forth a summary of our other income (loss) for each of the periods:

	Three Months Ended March 31,		Nine Months Ended March 31,	
	2025	2024	2025	2024
	(US\$'000)			
Other Income (Loss):				
Foreign currency transaction gain (loss)	\$ 11	\$ (118)	\$ (190)	\$ (22)
Interest income (expense), net	823	(4)	2,250	(16)
Other expense, net	—	(16)	(5)	(50)
Gain on extinguishment of liabilities	—	—	764	—
Unrealized loss on investment	—	—	—	(1)
Total other income (loss), net	\$ 834	\$ (138)	\$ 2,819	\$ (89)

Other income (loss), net during the three and nine months ended March 31, 2025, which consists of foreign currency transaction gain (loss), interest income (expense), other income (expense), gain on extinguishment of liabilities, and unrealized loss on investment, totaled \$834 thousand and \$2,819 thousand, respectively. Other income (loss), net during the three and nine months ended March 31, 2024, which consists of foreign currency transaction gain (loss), interest expense, other income (expense), and unrealized loss on investment, totaled \$(138) thousand and \$(89) thousand, respectively. Foreign currency transaction gains and losses reflect changes in foreign exchange rates. Net interest income for the three and nine months period ended March 31, 2025, in comparison to the loss reported for the three and nine months period ended March 31, 2024, reflects the increase in the Company's cash and cash equivalent balances. Other income (expense) recognized during the three and nine months ended March 31, 2025 relates to recognition of a franchise tax expenses. Gain on extinguishment of liabilities is due to the Company settling outstanding trade payables and accrued clinical development project costs of \$1.2 million with a vendor for \$495 thousand due to a contractual dispute regarding contract performance and deliverables. This settlement resulted in a gain of \$764 thousand in the current period.

Liquidity and Capital Resources

The Company has incurred cumulative losses and negative cash flows from operations since our predecessor's inception in 1995. The Company had accumulated losses of \$212 million as of March 31, 2025. We expect that our research and development expenses will increase due to the continued development of the OPMD program. It is also likely that there will be an increase in the general and administrative expenses due to the obligations of being a domestic public company in the United States.

We had no borrowings as of March 31, 2025 and do not currently have a credit facility.

As of March 31, 2025, we had cash and cash equivalents of approximately \$103.6 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our cash and cash equivalents are held in bank accounts. On October 11, 2024, we entered into the Sales Agreement as discussed above, which provides for the sale of up to \$75 million of our common stock from time-to-time in "at-the-market offerings". On March 25, 2025, the Company completed a financing which raised \$30 million.

The following table sets forth a summary of the net cash flow activity for each of the periods set forth below:

	Nine Months Ended	
	March 31,	
	2025	2024
	(US\$'000)	
Net cash provided by (used in):		
Operating activities	\$(15,375)	\$(16,110)
Investing activities	(18)	(179)
Financing activities	67,941	27,958
Effects of exchange rate changes on cash and cash equivalents	169	(3)
Net increase in cash, cash equivalents, and restricted cash	<u>\$ 52,717</u>	<u>\$ 11,666</u>

Operating activities

Net cash used in operating activities for the nine months ended March 31, 2025 and 2024 was \$15.4 million and \$16.1 million, respectively. Net cash used in operating activities was primarily the result of our net loss, partially offset by non-cash expenses, and changes in working capital, including a decrease in payables and increases trade and other receivables and prepaid expenses.

Investing activities

Net cash used in investing activities for the nine month periods ended March 31, 2025 and 2024 was \$18 thousand and \$179 thousand, respectively. Cash used in investing activities in the nine months ended March 31, 2025 and 2024 was related to the purchase of furniture and fixtures and lab equipment, respectively.

Financing activities

Net cash provided by financing activities was \$67.9 million and \$28.0 million for the nine months ended March 31, 2025 and 2024, respectively. Cash from financing activities in the nine months ended March 31, 2025 was related to the issuance of common stock from the exercise of pre-funded warrants, Series 2 warrants, and common warrants, and an underwritten and direct offering with net proceeds of \$67.9 million. Cash from financing activities in the nine months ended March 31, 2024 was related to the issuance of common stock, pre-funded warrants, and common warrants, with gross proceeds of \$30.9 million, partially offset by \$2.9 million in share issuance costs.

The future of the Company as an operating business will depend on its ability to manage operating costs and budgeted amounts and obtain adequate financing. While we continue to progress discussions and advance opportunities to engage with pharmaceutical companies and continue to seek licensing partners for ddRNAi in disease areas that are not our focus, there can be no assurance as to whether we will enter into such arrangements or what the terms of any such arrangement could be.

We do not have any products approved for sale and have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates.

Unless and until we establish significant revenues from licensing programs, strategic alliances or collaboration arrangements with pharmaceutical companies, or from product sales, we anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of product candidates and begin to prepare to commercialize any product that receives regulatory approval. We are subject to the risks inherent in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, complications, delays, and other unknown factors that may adversely affect our business. We estimate that our cash and cash equivalents will be sufficient to fund the Company's operations for at least the next twelve months from the date of this report.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development, and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the timing and costs of our clinical trials for our ddRNAi and silence and replace product candidates;
- the timing and costs of our preclinical studies for our ddRNAi and silence and replace product candidates;
- the number and characteristics of product candidates that we pursue;
- the outcome, timing, and costs of seeking regulatory approvals;
- revenue received from commercial sales of any of our product candidates that may receive regulatory approval;
- the terms and timing of any future collaborations, licensing, consulting, or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the extent to which we need to in-license or acquire other products and technologies.

Contractual Obligations and Commercial Commitments

On October 1, 2016, the Company entered into an operating lease for office space in Hayward, California that originally expired in April 2018. The Company has entered into lease amendments that extended the lease through December 2027. The Company also entered into a new lease in Los Angeles, California, which has an initial expiration date in July 2026. See Note 9 of the Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q.

The Company enters into contracts in the normal course of business with third-party contract research organizations, contract development and manufacturing organizations and other service providers and vendors. These contracts generally provide for termination on notice and, therefore, are cancellable contracts and not considered contractual obligations and commitments.

Critical Accounting Policies and Significant Accounting Estimates

The preparation of consolidated financial statements and related disclosures in conformity with accounting principles generally accepted in the United States of America requires management to make judgments, assumptions and estimates that affect the amounts reported. Note 2 of the Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q describes the significant accounting policies used in the preparation of the consolidated financial statements. Certain of these significant accounting policies are considered to be critical accounting policies.

A critical accounting policy is defined as one that is both material to the presentation of the Company's consolidated financial statements and requires management to make difficult, subjective, or complex judgments that could have a material effect on the Company's financial condition or results of operations. Specifically, these policies have the following attributes: (1) the Company is required to make assumptions about matters that are highly uncertain at the time of the estimate; and (2) different estimates the Company could reasonably have used, or changes in the estimate that are reasonably likely to occur, would have a material effect on the Company's financial condition or results of operations.

Estimates and assumptions about future events and their effects cannot be determined with certainty. The Company bases its estimates on historical experience and on various other assumptions believed to be applicable and reasonable under the circumstances. These estimates may change as new events occur, as additional information is obtained and as the Company's operating environment

changes. These changes have historically been minor and have been included in the consolidated financial statements as soon as they became known. In addition, management is periodically faced with uncertainties, the outcomes of which are not within its control and will not be known for prolonged periods of time. These uncertainties are discussed in the section above entitled “Risk Factors.” Based on a critical assessment of its accounting policies and the underlying judgments and uncertainties affecting the application of those policies, management believes that the Company’s consolidated financial statements are fairly stated in accordance with accounting principles generally accepted in the United States of America and provide a meaningful presentation of the Company’s financial condition and results of operations.

Management believes that the following are critical accounting policies:

Research and Development Expense

Research and development expenses relate primarily to the cost of conducting clinical and preclinical trials. Preclinical and clinical development costs are a significant component of research and development expenses. The Company records accrued liabilities for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and includes these costs in trade and other payables on the consolidated balance sheets and within research and development expenses on the consolidated statements of operations and comprehensive loss.

The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance at the end of each reporting period. As actual costs become known, the Company adjusts its accrued liabilities. The Company has not experienced any material differences between accrued costs and actual costs incurred.

Share-based Compensation Expense

The Company records share-based compensation in accordance with ASC 718, *Stock Compensation*. ASC 718 requires the fair value of all share-based employee compensation awarded to employees and non-employees to be recorded as an expense over the shorter of the service period or the vesting period. The Company determines employee and non-employee share-based compensation based on grant-date fair value using the Black-Scholes Option Pricing Model.

Recent Accounting Pronouncements

Accounting Standards recently adopted

ASU 2016-13 – In June 2016, the FASB issued ASU No. 2016-13: “*Financial Instruments-Credit Losses (Topic 326)*”. This ASU represents a significant change in the accounting for credit losses model by requiring immediate recognition of management’s estimates of current expected credit losses (CECL). Under the prior model, losses were recognized only as they were incurred. The Company adopted this ASU effective July 1, 2023 and determined that its impact on the accompanying consolidated financial statements is immaterial.

Recently Issued Accounting Standards not yet adopted

In December 2023, the FASB issued ASU No. 2023-09, “*Income Taxes (Topic 740) – Improvements to Income Tax Disclosures*”, which enhances the transparency, effectiveness and comparability of income tax disclosures by requiring consistent categories and greater disaggregation of information related to income tax rate reconciliations and the jurisdictions in which income taxes are paid. This guidance is effective for annual periods beginning after December 15, 2024 with early adoption permitted. The Company is currently evaluating the impact of the ASU on its income tax disclosures within the consolidated financial statements.

In November 2023, the FASB issued ASU No. 2023-07, “*Segment Reporting (Topic 280) – Improvements to Reportable Segment Disclosures*”, which improves reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. This ASU also expands disclosure requirements to enable users of financial statements to better understand the entity’s measurement and assessment of segment performance and resource allocation. This guidance is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The Company is currently evaluating the impact of the ASU on its disclosures within the consolidated financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

As a smaller reporting company, we are not required to provide the information pursuant to this Item.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We have established disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended). As of the end of the period covered by this Report we carried out an evaluation under the supervision and with the participation of our management, including our principal executive officer and principal financial and accounting officer, of the effectiveness of our disclosure controls and procedures pursuant to Rule 13a-15 of the Securities and Exchange Act of 1934, as amended. Based on this evaluation, and as a result of the material weakness in our internal control over financial reporting further described in *Management's Report on Internal Control Over Financial Reporting* in Item 9A of our Form 10-K for the fiscal year ended June 30, 2024 (relating to our accounting personnel not being able to process and account for complex, non-routine transactions), in accordance with US GAAP due to the Company lacking sufficient personnel and outside consultants with technical accounting expertise to process and account for complex and non-routine transactions), our principal executive officer and principal financial officer, concluded that as of March 31, 2025, our disclosure controls and procedures were not effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer as appropriate to allow timely decisions regarding required disclosure. In order to remediate this matter, we have reviewed our accounting team and have engaged the services of additional accounting experts to assist in the accounting and reporting of complex, non-routine transactions. We will consider the material weakness to be fully remediated once the applicable controls operate for a sufficient period of time and our management has concluded, through testing, that these controls are operating effectively. We have established the process of identifying these transactions and adding additional internal reviews to account for them under current accounting standards. These processes will be tested in the coming months.

We do not expect that our disclosure controls and procedures or our internal controls will prevent all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected.

Changes in Internal Control over Financial Reporting

Other than the efforts towards remediating the material weakness as previously described above, there were no changes in our internal controls over financial reporting during the quarter ended March 31, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II
OTHER INFORMATION

Item 1. Legal Proceedings

We are currently not a party to any material legal proceedings.

Item 1A. Risk Factors

There have been no material changes to the risk factors disclosed in Item 1A of the Company's Annual Report on Form 10-K for the fiscal year ended June 30, 2024, and in the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended December 31, 2024.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

During the quarter ended March 31, 2025, none of our directors or officers adopted, modified, or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement" as such terms are defined under Item 408 of Regulation S-K.

Item 6. Exhibits.

Number	Description of Document
4.1	<u>Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K filed on March 26, 2025)</u>
10.1	<u>Underwriting Agreement, dated March 25, 2025, by and between Benitec Biopharma Inc., Leerink Partners LLC and TD Securities (USA) LLC (incorporated by reference to Exhibit 1.1 to the Company's Current Report on Form 8-K filed on March 26, 2025)</u>
10.2	<u>Securities Purchase Agreement, dated March 25, 2025, by and between Benitec Biopharma Inc., Averill Master Fund, Ltd. and Averill Madison Master Fund, Ltd. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 26, 2025)</u>
10.3	<u>Registration Rights Agreement, dated March 26, 2025, by and between Benitec Biopharma Inc., Averill Master Fund, Ltd. and Averill Madison Master Fund (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 27, 2025)</u>
10.4	<u>Fourth Amendment to Lease, dated August 30, 2021, by and between Hayward Point Eden I Limited Partnership and Benitec Biopharma Inc.*</u>
10.5	<u>Fifth Amendment to Lease, dated February 24, 2025, by and between Hayward Point Eden I Limited Partnership and Benitec Biopharma Inc.*</u>
31.1	<u>Statement of CEO Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002*</u>
31.2	<u>Statement of CFO Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002*</u>
32.1	<u>Statement of CEO Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**</u>
32.2	<u>Statement of CFO Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**</u>
101.INS	Inline XBRL Instance Document*
101.SCH	Inline XBRL Taxonomy Extension Schema Document*
101.CAL	Inline XBRL Calculation Linkbase Document*
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document*
101.LAB	Inline XBRL Label Linkbase Document*
101.PRE	Inline XBRL Taxonomy Presentation Linkbase Document*
104	Cover Page Interactive Data File—the cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document

* Filed herewith.

** Furnished, not filed.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on our behalf by the undersigned thereunto duly authorized.

Dated: May 14, 2025

Benitec Biopharma Inc.

/s/ Jerel Banks

Jerel Banks
Executive Chairman and Chief Executive Officer
(principal executive officer)

/s/ Megan Boston

Megan Boston
Chief Financial Officer and Secretary (principal financial and
accounting officer)

FOURTH AMENDMENT TO LEASE

This FOURTH AMENDMENT TO LEASE ("**Fourth Amendment**") is made and entered into as of August 30, 2021, by and between HAYWARD POINT EDEN I LIMITED PARTNERSHIP, a Delaware limited partnership ("**Landlord**"), and BENITEC BIOPHARMA INC., a Delaware corporation ("**Tenant**").

RECITALS:

A. Landlord and Tenant (as successor-in-interest to Benitec Biopharma LTD, an Australia registered corporation) are parties to that certain Lease dated May 12, 2014 (the "**Original Lease**"), as amended by that certain First Amendment to Lease dated May 7, 2015 (the "**First Amendment**"), that certain Second Amendment to Lease dated April 26, 2018 (The "**Second Amendment**"), and that certain Third Amendment to Lease dated January 10, 2019 (the "**Third Amendment**"), whereby Tenant currently leases 7,295 rentable square feet of space (the "**Premises**") in the building (the "**Building**") commonly known as Building B, which Premises is comprised of (i) 4,754 rentable square feet of space with a street address of 3940 Trust Way, Hayward, California 94545, and (ii) 2,541 rentable square feet of space with a street address of 3942 Trust Way in the Building, all located in the office project known as "Point Eden Business Park" (the "**Project**"). The Lease, the First Amendment, the Second Amendment, and the Third Amendment are, collectively, the "**Lease**."

B. The parties desire to amend the Lease on the terms and conditions set forth in this Fourth Amendment.

AGREEMENT:

NOW, THEREFORE, in consideration of the foregoing recitals and the mutual covenants contained herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto hereby agree as follows:

1. **Terms.** All capitalized terms when used herein shall have the same respective meanings as are given such terms in the Lease unless expressly provided otherwise in this Fourth Amendment.
2. **Condition of the Premises.** Landlord and Tenant acknowledge that Tenant has been occupying the Premises pursuant to the Lease, and therefore Tenant continues to accept the Premises in its presently existing, "as is" condition. Landlord shall not be obligated to provide or pay for any improvement work or services related to the improvement of the Premises, provided that Landlord shall perform an inspection of the Building Systems serving the Premises (excluding any specialty or supplemental Building Systems installed by or for Tenant) and the structural portions of the Building (including the roof and roof membrane) and perform repairs as reasonably necessary such that the same are in good working order and condition, and nothing contained herein shall limit or modify Landlord's repair and maintenance obligations currently set forth in the Lease. Tenant also acknowledges that neither Landlord nor any agent of Landlord has made any representation or warranty regarding the condition of the Premises, the Building, or the Project or with respect to the suitability of the same for the conduct of Tenant's business.

3. Lease Term.

3.1 **Second Extended Lease Term.** Pursuant to the Lease, the Lease Term is scheduled to expire on June 14, 2022. Landlord and Tenant hereby agree to extend the Lease Term for a period of three (3) years, from June 15, 2022, through June 14, 2025 (the “**Second Extended Term**”), on the terms and conditions set forth in the Lease, as hereby amended by this Fourth Amendment, unless sooner terminated as provided in the Lease.

3.2 **Option to Extend Lease Term.** Landlord and Tenant acknowledge and agree that Tenant shall continue to have one (1) option to extend the Lease Term for a period of three (3) years in accordance with, and pursuant to the terms of, Section 2.2 of the Original Lease, Section 8 of the First Amendment and Section 3.2 of the Second Amendment; provided, however, all references therein to the “initial Lease Term” shall be deemed to refer to the “Second Extended Term”.

4. Rent.

4.1 **Base Rent.** During the Second Extended Term, Tenant shall pay monthly installments of Base Rent for the Premises as follows, and otherwise shall pay Base Rent in accordance with the terms of the Lease:

Period During Second Extended Term	Annual Base Rent	Monthly Installment of Base Rent	Monthly Rental Rate per Square Foot
June 15, 2022- June 14, 2023	\$284,505.00	\$23,708.75	\$ 3.25
June 15, 2023- June 14, 2024	\$294,462.68	\$24,538.56	\$ 3.36
June 15, 2024- June 14, 2025	\$304,768.87	\$25,397.41	\$ 3.48

4.2 **Direct Expenses.** Prior to and continuing throughout the Second Extended Term, Tenant shall pay Tenant’s Share of all Direct Expenses which arise or accrue with respect to the Premises during such period in accordance with the terms of the Lease.

5. **Broker.** Landlord and Tenant hereby warrant to each other that they have had no dealings with any real estate broker or agent in connection with the negotiation of this Fourth Amendment other than CBRE, Inc. (the “**Broker**”), and that they know of no other real estate broker or agent who is entitled to a commission in connection with this Fourth Amendment. Each party agrees to indemnify and defend the other party against and hold the other party harmless from any and all claims, demands, losses, liabilities, lawsuits, judgments, costs and expenses (including without limitation reasonable attorneys’ fees) with respect to any leasing commission or equivalent compensation alleged to be owing on account of any dealings with any real estate broker or agent, other than the Broker, occurring by, through, or under the indemnifying party. The terms of this Section 5 shall survive the expiration or earlier termination of the term of the Lease, as hereby amended.

6. **Security Deposit.** Notwithstanding anything in the Lease to the contrary, the Security Deposit held by Landlord pursuant to Article 21 of the Original Lease, as amended hereby, shall equal \$25,397.41. Landlord and Tenant acknowledge that, in accordance with the Lease, Tenant has previously delivered the sum \$9,165.54 (the “**Existing Security Deposit**”) to Landlord as security for the faithful performance by Tenant of the terms, covenants and conditions of the Lease. Concurrently with Tenant’s execution of this Fourth Amendment, Tenant shall deposit with Landlord an amount equal to \$16,231.87 to be held by Landlord as a part of the Security Deposit. To the extent that the total amount held by Landlord at any time as security for the Lease, as hereby amended, is less than \$25,397.41, Tenant shall pay the difference to Landlord in accordance with the terms of the Lease.

7. **California Required Disclosures.** For purposes of Section 1938 of the California Civil Code, Landlord hereby discloses to Tenant, and Tenant hereby acknowledges, that the Project, Building and Premises have not undergone inspection by a Certified Access Specialist (CASP).

8. **Judicial Reference.** NOTWITHSTANDING ANY PROVISION TO THE CONTRARY CONTAINED IN THE LEASE, THE PARTIES HEREBY WAIVE, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THE RIGHT TO TRIAL BY JURY IN ANY LITIGATION ARISING OUT OF OR RELATING TO THE LEASE. IF THE JURY WAIVER PROVISIONS OF THE LEASE ARE NOT ENFORCEABLE UNDER CALIFORNIA LAW, THEN THE FOLLOWING PROVISIONS SHALL APPLY. IT IS THE DESIRE AND INTENTION OF THE PARTIES TO AGREE UPON A MECHANISM AND PROCEDURE UNDER WHICH CONTROVERSIES AND DISPUTES ARISING OUT OF THE LEASE OR RELATED TO THE PREMISES WILL BE RESOLVED IN A PROMPT AND EXPEDITIOUS MANNER. ACCORDINGLY, EXCEPT WITH RESPECT TO ACTIONS FOR UNLAWFUL OR FORCIBLE DETAINER OR WITH RESPECT TO THE PREJUDGMENT REMEDY OF ATTACHMENT, ANY ACTION, PROCEEDING OR COUNTERCLAIM BROUGHT BY EITHER PARTY HERETO AGAINST THE OTHER (AND/OR AGAINST ITS OFFICERS, DIRECTORS, EMPLOYEES, AGENTS OR SUBSIDIARIES OR AFFILIATED ENTITIES) ON ANY MATTERS WHATSOEVER ARISING OUT OF OR IN ANY WAY CONNECTED WITH THE LEASE, TENANT’S USE OR OCCUPANCY OF THE PREMISES AND/OR ANY CLAIM OF INJURY OR DAMAGE, WHETHER SOUNDING IN CONTRACT, TORT, OR OTHERWISE, SHALL BE HEARD AND RESOLVED BY A REFEREE UNDER THE PROVISIONS OF THE CALIFORNIA CODE OF CIVIL PROCEDURE, SECTIONS 638 -645.1, INCLUSIVE (AS SAME MAY BE AMENDED, OR ANY SUCCESSOR STATUTE(S) THERETO) (THE “**REFEREE SECTIONS**”). ANY FEE TO INITIATE THE JUDICIAL REFERENCE PROCEEDINGS AND ALL FEES CHARGED AND COSTS INCURRED BY THE REFEREE SHALL BE PAID BY THE PARTY INITIATING SUCH PROCEDURE (EXCEPT THAT IF A REPORTER IS REQUESTED BY EITHER PARTY, THEN A

REPORTER SHALL BE PRESENT AT ALL PROCEEDINGS WHERE REQUESTED AND THE FEES OF SUCH REPORTER—EXCEPT FOR COPIES ORDERED BY THE OTHER PARTIES – SHALL BE BORNE BY THE PARTY REQUESTING THE REPORTER); PROVIDED HOWEVER, THAT ALLOCATION OF THE COSTS AND FEES, INCLUDING ANY INITIATION FEE, OF SUCH PROCEEDING SHALL BE ULTIMATELY DETERMINED IN ACCORDANCE WITH SECTION 29.21 OF THE ORIGINAL LEASE. THE VENUE OF THE PROCEEDINGS SHALL BE IN THE COUNTY IN WHICH THE PREMISES ARE LOCATED. WITHIN TEN (10) DAYS OF RECEIPT BY ANY PARTY OF A WRITTEN REQUEST TO RESOLVE ANY DISPUTE OR CONTROVERSY PURSUANT TO THIS SECTION 8, THE PARTIES SHALL AGREE UPON A SINGLE REFEREE WHO SHALL TRY ALL ISSUES, WHETHER OF FACT OR LAW, AND REPORT A FINDING AND JUDGMENT ON SUCH ISSUES AS REQUIRED BY THE REFEREE SECTIONS. IF THE PARTIES ARE UNABLE TO AGREE UPON A REFEREE WITHIN SUCH TEN (10) DAY PERIOD, THEN ANY PARTY MAY THEREAFTER FILE A LAWSUIT IN THE COUNTY IN WHICH THE PREMISES ARE LOCATED FOR THE PURPOSE OF APPOINTMENT OF A REFEREE UNDER THE REFEREE SECTIONS. IF THE REFEREE IS APPOINTED BY THE COURT, THE REFEREE SHALL BE A NEUTRAL AND IMPARTIAL RETIRED JUDGE WITH SUBSTANTIAL EXPERIENCE IN THE RELEVANT MATTERS TO BE DETERMINED, FROM JAMS, THE AMERICAN ARBITRATION ASSOCIATION OR SIMILAR MEDIATION/ARBITRATION ENTITY. THE PROPOSED REFEREE MAY BE CHALLENGED BY ANY PARTY FOR ANY OF THE GROUNDS LISTED IN THE REFEREE SECTIONS. THE REFEREE SHALL HAVE THE POWER TO DECIDE ALL ISSUES OF FACT AND LAW AND REPORT ITS OR HER DECISION ON SUCH ISSUES, AND TO ISSUE ALL RECOGNIZED REMEDIES AVAILABLE AT LAW OR IN EQUITY FOR ANY CAUSE OF ACTION THAT IS BEFORE THE REFEREE, INCLUDING AN AWARD OF ATTORNEYS' FEES AND COSTS IN ACCORDANCE WITH THE LEASE. THE REFEREE SHALL NOT, HOWEVER, HAVE THE POWER TO AWARD PUNITIVE DAMAGES, NOR ANY OTHER DAMAGES WHICH ARE NOT PERMITTED BY THE EXPRESS PROVISIONS OF THE LEASE, AND THE PARTIES HEREBY WAIVE ANY RIGHT TO RECOVER ANY SUCH DAMAGES. THE PARTIES SHALL BE ENTITLED TO CONDUCT ALL DISCOVERY AS PROVIDED IN THE CALIFORNIA CODE OF CIVIL PROCEDURE, AND THE REFEREE SHALL OVERSEE DISCOVERY AND MAY ENFORCE ALL DISCOVERY ORDERS IN THE SAME MANNER AS ANY TRIAL COURT JUDGE, WITH RIGHTS TO REGULATE DISCOVERY AND TO ISSUE AND ENFORCE SUBPOENAS, PROTECTIVE ORDERS AND OTHER LIMITATIONS ON DISCOVERY AVAILABLE UNDER CALIFORNIA LAW. THE REFERENCE PROCEEDING SHALL BE CONDUCTED IN ACCORDANCE WITH CALIFORNIA LAW (INCLUDING THE RULES OF EVIDENCE), AND IN ALL REGARDS, THE REFEREE SHALL FOLLOW CALIFORNIA LAW APPLICABLE AT THE TIME OF THE REFERENCE PROCEEDING. THE PARTIES SHALL PROMPTLY AND DILIGENTLY COOPERATE WITH ONE ANOTHER AND THE REFEREE, AND SHALL PERFORM SUCH ACTS AS MAY BE NECESSARY TO OBTAIN A PROMPT AND EXPEDITIOUS RESOLUTION OF THE DISPUTE OR CONTROVERSY IN ACCORDANCE WITH THE TERMS OF TIDS SECTION 8. IN TIDS REGARD, THE PARTIES AGREE THAT THE PARTIES AND THE REFEREE SHALL USE BEST EFFORTS TO ENSURE THAT (A) DISCOVERY BE CONDUCTED FOR A PERIOD NO LONGER THAN SIX (6) MONTHS FROM THE DATE THE REFEREE IS APPOINTED, EXCLUDING

MOTIONS REGARDING DISCOVERY, AND (B) A TRIAL DATE BE SET WITHIN NINE (9) MONTHS OF THE DATE THE REFEREE IS APPOINTED. IN ACCORDANCE WITH SECTION 644 OF THE CALIFORNIA CODE OF CIVIL PROCEDURE, THE DECISION OF THE REFEREE UPON THE WHOLE ISSUE MUST STAND AS THE DECISION OF THE COURT, AND UPON THE FILING OF THE STATEMENT OF DECISION WITH THE CLERK OF THE COURT, OR WITH THE JUDGE IF THERE IS NO CLERK, JUDGMENT MAY BE ENTERED THEREON IN THE SAME MANNER AS IF THE ACTION HAD BEEN TRIED BY THE COURT. ANY DECISION OF THE REFEREE AND/OR JUDGMENT OR OTHER ORDER ENTERED THEREON SHALL BE APPEALABLE TO THE SAME EXTENT AND IN THE SAME MANNER THAT SUCH DECISION, JUDGMENT, OR ORDER WOULD BE APPEALABLE IF RENDERED BY A JUDGE OF THE SUPERIOR COURT IN WHICH VENUE IS PROPER HEREUNDER. THE REFEREE SHALL IN HIS/HER STATEMENT OF DECISION SET FORTH HIS/HER FINDINGS OF FACT AND CONCLUSIONS OF LAW. THE PARTIES INTEND THIS GENERAL REFERENCE AGREEMENT TO BE SPECIFICALLY ENFORCEABLE IN ACCORDANCE WITH THE CODE OF CNIL PROCEDURE. NOTHING IN THIS SECTION 8 SHALL PREJUDICE THE RIGHT OF ANY PARTY TO OBTAIN PROVISIONAL RELIEF OR OTHER EQUITABLE REMEDIES FROM A COURT OF COMPETENT JURISDICTION AS SHALL OTHERWISE BE AVAILABLE UNDER THE CODE OF CIVIL PROCEDURE AND/OR APPLICABLE COURT RULES.

9. **No Further Modification**. Except as specifically set forth in this Fourth Amendment, all of the terms and provisions of the Lease shall remain unmodified and in full force and effect.

[SIGNATURES FOLLOW ON NEXT PAGE]

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[Fourth Amendment]
[Benitec Biopharma LTD]

IN WITNESS WHEREOF, this Fourth Amendment has been executed as of the day and year first above written.

“LANDLORD”

HAYWARD POINT EDEN I LIMITED
PARTNERSHIP,
a Delaware limited partnership

By: HCP Point Eden Incorporated,
a Delaware corporation,
its General Partner

By: /s/ Scott Bohn

Name: Scott Bohn

Its: Senior Vice President

“TENANT”

BENITEC BIOPHARMA INC.,
a Delaware corporation,

By: /s/ Jerel A. Banks

Name: Jerel A. Banks, M.D. Ph.d.

Its: Executive Chairman and CEO

By its execution of this Fourth Amendment, the undersigned, guarantor under the Lease, hereby consents to the foregoing Fourth Amendment, and hereby reaffirms its obligations pursuant to that certain Guaranty of Lease, dated of even date with the Original Lease, with respect to the Lease, as amended by this Fourth Amendment.

“GUARANTOR”

TACERE THERAPEUTICS, INC.,
a Delaware corporation

BY: /s/ Jerel A. Banks

Print Name: Jerel A. Banks, M.D., Ph.D.

Its: Director

FIFTH AMENDMENT TO LEASE

This FIFTH AMENDMENT TO LEASE ("**Fifth Amendment**") is made and entered into as of 2/24/2025 (the "**Fifth Amendment Effective Date**"), by and between HAYWARD POINT EDEN I LIMITED PARTNERSHIP, a Delaware limited partnership ("**Landlord**"), and BENITEC BIOPHARMA INC., a Delaware corporation ("**Tenant**").

RECITALS:

A. Landlord and Tenant (as successor-in-interest to Benitec Biopharma LTD, an Australia registered corporation) are parties to that certain Lease dated May 12, 2014 (the "**Original Lease**"), as amended by that certain (i) First Amendment to Lease dated May 7, 2015 (the "**First Amendment**"), (ii) Second Amendment to Lease dated April 26, 2018 (the "**Second Amendment**"), (iii) Third Amendment to Lease dated January 10, 2019 (the "**Third Amendment**") and (iv) Fourth Amendment to Lease dated August 30, 2021 (the "**Fourth Amendment**"; and together with the Original Lease, First Amendment, Second Amendment and Third Amendment, the "**Lease**"), whereby Tenant currently leases 7,295 rentable square feet of space (the "**Premises**") in the building (the "**Building**") commonly known as Building B, which Premises is comprised of (i) 4,754 rentable square feet of space with a street address of 3940 Trust Way, Hayward, California 94545, and (ii) 2,541 rentable square feet of space with a street address of 3942 Trust Way in the Building, all located in the office project known as "**Point Eden Business Park**" (the "**Project**").

B. The parties desire to amend the Lease on the terms and conditions set forth in this Fifth Amendment.

C. Landlord's name was incorrectly listed on the cover page of the Lease as "HCP LS REDWOOD CITY, LLC, a Delaware limited liability company".

AGREEMENT:

NOW, THEREFORE, in consideration of the foregoing recitals and the mutual covenants contained herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto hereby agree as follows:

1. **Terms.** All capitalized terms when used herein shall have the same respective meanings as are given such terms in the Lease unless expressly provided otherwise in this Fifth Amendment.

2. **Condition of the Premises.** Landlord and Tenant acknowledge that Tenant has been occupying the Premises pursuant to the Lease, and therefore Tenant continues to accept the Premises in its presently existing, "as is" condition. Landlord shall not be obligated to provide or pay for any improvement work or services related to the improvement of the Premises, provided nothing contained herein shall limit or modify Landlord's repair and maintenance obligations currently set forth in the Lease. Tenant also acknowledges that neither Landlord nor any agent of Landlord has made any representation or warranty regarding the condition of the Premises, the Building, or the Project or with respect to the suitability of the same for the conduct of Tenant's business.

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3. Lease Term.

3.1. **Third Extended Lease Term.** Pursuant to the Lease, the Second Extended Term is scheduled to expire on June 14, 2025. Landlord and Tenant hereby agree to extend the Lease Term for a period of thirty (30) months, from June 15, 2025 (the “**Third Extended Term Commencement Date**”), through December 14, 2027 (the “**Third Extended Term**”), on the terms and conditions set forth in the Lease, as hereby amended by this Fifth Amendment (the “**Amended Lease**”), unless sooner terminated as provided in the Amended Lease.

3.2. **Option to Extend Lease Term.** Landlord and Tenant acknowledge and agree that Tenant shall continue to have one (1) option to extend the Third Extended Term for a period of three (3) years in accordance with, and pursuant to the terms of, Section 2.2 of the Original Lease, Section 8 of the First Amendment, Section 3.2 of the Second Amendment and Section 3.2 of the Fourth Amendment (collectively, the “**Option Provisions**”); provided, however, (i) all references in the Option Provisions to the “initial Lease Term” shall be deemed to refer to the “Third Extended Term”, and (ii) all references in the Option Provisions to the “Lease” shall be deemed to refer to the “Amended Lease”.

4. Rent.

4.1. **Base Rent.** Commencing on the Third Extended Term Commencement Date and continuing during the Third Extended Term, Tenant shall pay monthly installments of Base Rent for the Premises as follows, and otherwise shall pay Base Rent in accordance with the terms of the Lease:

Period During Third Extended Term	Annual Base Rent	Monthly Installment of Base Rent	Approximate Monthly Rental Rate per Square Foot
June 15, 2025 – June 14, 2026	\$328,275.00*	\$27,356.25	\$ 3.75
June 15, 2026 – June 14, 2027	\$339,764.63	\$28,313.72	\$ 3.88
June 15, 2027 – December 14, 2027	\$351,656.39	\$29,304.70	\$ 4.02

* Notwithstanding the foregoing, Base Rent shall be abated for the Fifth Amendment Rent Abatement Period as further described in Section 4.2, below.

4.2. **Abated Base Rent.** Provided that Tenant is not then in default of the Amended Lease, then during the period commencing on the first full calendar month following the Third Extended Term Commencement Date and ending on fourth (4th) full calendar month following the Third Extended Term Commencement Date (i.e., the months of July, August, September and October 2025) (the “**Fifth Amendment Rent Abatement Period**”), Tenant shall not be obligated to pay any Base Rent otherwise attributable to the Premises during such Fifth

Amendment Rent Abatement Period (the “**Fifth Amendment Rent Abatement**”). Landlord and Tenant acknowledge that the aggregate amount of the Fifth Amendment Rent Abatement equals \$109,425.00 (*i.e.*, \$27,356.25 per month). Tenant acknowledges and agrees that the foregoing Fifth Amendment Rent Abatement has been granted to Tenant as additional consideration for entering into this Fifth Amendment, and for agreeing to pay the Rent and perform the terms and conditions otherwise required under the Amended Lease. If Tenant shall be in default under the Amended Lease and shall fail to cure such default within the notice and cure period, if any, permitted for cure pursuant to the Amended Lease, or if the Amended Lease is terminated for any reason, other than as the result of casualty or condemnation, then Landlord may at its option, by notice to Tenant, elect, in addition to any other remedies Landlord may have under the Amended Lease, one or both of the following remedies: (i) that Tenant shall immediately become obligated to pay to Landlord all Base Rent abated hereunder during the Fifth Amendment Rent Abatement Period, with interest as provided pursuant to the Lease from the date such Base Rent would have otherwise been due but for the abatement provided herein, or (ii) that the dollar amount of the unapplied portion of the Fifth Amendment Rent Abatement as of such default or termination shall be converted to a credit to be applied to the Base Rent applicable at the end of the Fifth Extended Term and Tenant shall immediately be obligated to begin paying Base Rent for the Premises in full.

4.3. **Direct Expenses.** Prior to and continuing throughout the Third Extended Term, Tenant shall pay Tenant’s Share of all Direct Expenses which arise or accrue with respect to the Premises during such period in accordance with the terms of the Amended Lease.

5. **Utility Information.** Tenant hereby acknowledges and agrees that (i) pursuant to certain applicable laws and/or sustainability reporting requirements (collectively the “**Energy Disclosure Requirements**”), Landlord may be required to disclose factual information concerning Tenant’s energy usage at the Project to certain third parties, including, without limitation, prospective purchasers, lenders and tenants of the Building and/or Landlord’s consultants and/or vendors (the “**Tenant Energy Use Disclosure**”), and (ii) in connection therewith, to the extent any utilities are separately metered and paid by Tenant, Tenant shall cooperate with Landlord as necessary to submit energy and water consumption data, including total usage and total charges as they appear on Tenant’s electric, gas, water, and other utility bills, in a format deemed reasonably acceptable by Landlord. Tenant hereby further (A) consents to all such Tenant Energy Use Disclosures made by Landlord, (B) acknowledges that Landlord shall not be required to notify Tenant of any Tenant Energy Use Disclosure, and (C) releases Landlord from any and all losses, costs, damages, expenses and liabilities relating to, arising out of and/or resulting from any Tenant Energy Use Disclosure made in accordance with the foregoing provisions. The terms of this Section 5 shall survive the expiration or earlier termination of the Amended Lease.

6. **Broker.** Landlord and Tenant hereby warrant to each other that they have had no dealings with any real estate broker or agent in connection with the negotiation of this Fifth Amendment other than CBRE, Inc., dually representing Landlord and Tenant (the “**Broker**”), and that they know of no other real estate broker or agent who is entitled to a commission in connection with this Fifth Amendment. The fees of Broker shall be paid by Landlord pursuant to the terms of a separate agreement between Landlord and Broker. Each party agrees to indemnify and defend the other party against and hold the other party harmless from any and all claims, demands, losses, liabilities, lawsuits, judgments, costs and expenses (including without limitation reasonable attorneys’ fees) with respect to any leasing commission or equivalent compensation alleged to be owing on account of any dealings with any real estate broker or agent, other than the Broker, occurring by, through, or under the indemnifying party. The terms of this Section 6 shall survive the expiration or earlier termination of the term of the Lease, as hereby amended.

7. **California Required Disclosures**. For purposes of Section 1938 of the California Civil Code, Landlord hereby discloses to Tenant, and Tenant hereby acknowledges, that the Project, Building and Premises have not undergone inspection by a Certified Access Specialist (CAsp).

8. **Confidentiality**. Tenant acknowledges that the terms and conditions of this Fifth Amendment are to remain confidential for Landlord's benefit, and may not be disclosed by Tenant to anyone, (other than on a need to know basis to Tenant's financial, legal and actual and prospective lenders and investors) by any manner or means, directly or indirectly, without Landlord's prior written consent; however, Tenant may disclose the terms and conditions of this Fifth Amendment if required by law or court order and in any litigation between the parties. Tenant shall be liable for any disclosures made in violation of this **Section 8** by Tenant or by any entity or individual to whom the terms of and conditions of this Amendment were disclosed or made available by Tenant. The consent by Landlord to any disclosures shall not be deemed to be a waiver on the part of Landlord of any prohibition against any future disclosure.

9. **Limitation on Liability**. Notwithstanding anything to the contrary set forth in the Amended Lease, neither Landlord and Landlord Parties nor Tenant (except for Tenant's obligations under **Sections 5.3 and 16** of the Original Lease) shall be liable to the other for any consequential, special or punitive damages of any kind, in each case, however occurring.

10. **REIT Provision**. Notwithstanding anything contained in the Amended Lease to the contrary, Tenant shall not: (i) make a transfer to an entity in which, under the Internal Revenue Code of 1986, as amended (the "**Code**"), any entity that directly or indirectly owns Landlord and is qualified as a real estate investment trust (a "**REIT Owner**") owns, directly, indirectly or by applying constructive ownership rules set forth in Section 856(d)(5) of the Code, a ten percent (10%) or greater interest; or (ii) make any transfer or other action under **Section 14.8** of the Original Lease, in a manner that would cause any portion of the amounts received by Landlord pursuant to the Amended Lease, to fail to qualify as "rents from real property" within the meaning of Section 856(d) of the Code.

11. **Landlord's Notice Address**. Effective as of the Fifth Amendment Effective Date, Landlord's address for notices under **Section 29.18** of the Original Lease shall be revised to be:

Hayward Point Eden I Limited Partnership
c/o Healthpeak Properties, Inc.
1900 Main Street, 5th Floor
Irvine, CA 92614
Attn: LS Asset Management

with a copy to:

Hayward Point Eden I Limited Partnership
c/o Healthpeak Properties, Inc.
1900 Main Street, 5th Floor
Irvine, CA 92614
Attn: Legal Department

and

Allen Matkins Leck Gamble Mallory & Natsis LLP
TCW Tower
865 S Figueroa St, Suite 2800
Los Angeles, CA 90017
Attn: Steven M. Farenbaugh, Esq.

12. **No Further Modification**. Except as specifically set forth in this Fifth Amendment, all of the terms and provisions of the Lease shall remain unmodified and in full force and effect.

[SIGNATURES FOLLOW ON NEXT PAGE]

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IN WITNESS WHEREOF, this Fifth Amendment has been executed as of the day and year first above written.

“LANDLORD”

HAYWARD POINT EDEN I LIMITED PARTNERSHIP,
a Delaware limited partnership

By: HCP Point Eden Incorporated,
a Delaware corporation,
its General Partner

By: /s/ Scott Bohn

Name: Scott Bohn

Its: Chief Development Officer

“TENANT”

BENITEC BIOPHARMA INC.,
a Delaware corporation

By: /s/ Jerel Banks

Name: Jerel Banks

Its: Executive Chairman and CEO

By its execution of this Fifth Amendment, the undersigned, guarantor under the Lease, hereby consents to the foregoing Fifth Amendment, and hereby reaffirms its obligations pursuant to that certain Guaranty of Lease, dated of even date with the Original Lease, with respect to the Lease, as amended by this Fifth Amendment.

“GUARANTOR”

TACERE THERAPEUTICS, INC.,
a Delaware corporation

By: /s/ Jerel Banks

Print Name: Jerel Banks

Its: Executive Chairman and CEO

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Statement Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 by
Principal Executive Officer
Regarding Facts and Circumstances Relating to Exchange Act Filings

I, Jerel Banks, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Benitec Biopharma Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 14, 2025

/s/ Jerel Banks

Jerel Banks

Executive Chairman and Chief Executive Officer

Statement Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 by
Principal Financial Officer
Regarding Facts and Circumstances Relating to Exchange Act Filings

I, Megan Boston, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Benitec Biopharma Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 14, 2025

/s/ Megan Boston

Megan Boston
Chief Financial Officer and Secretary (principal financial
and accounting officer)

Statement Pursuant to Section 906 the Sarbanes-Oxley Act of 2002
By
Principal Executive Officer
Regarding Facts and Circumstances Relating to Exchange Act Filings

Dated: May 14, 2025

I, Jerel Banks, Chief Executive Officer of Benitec Biopharma Inc., hereby certify, to my knowledge, that:

1. the accompanying Quarterly Report on Form 10-Q of Benitec Biopharma Inc. for the three month period ended March 31, 2025 (the “Report”) fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities and Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of Benitec Biopharma Inc.

IN WITNESS WHEREOF, the undersigned has executed this Statement as of the date first written above.

/s/ Jerel Banks

Jerel Banks

Executive Chairman and Chief Executive Officer

Statement Pursuant to Section 906 the Sarbanes-Oxley Act of 2002
By
Principal Financial Officer
Regarding Facts and Circumstances Relating to Exchange Act Filings

Dated: May 14, 2025

I, Megan Boston, Chief Financial Officer (principal accounting officer) of Benitec Biopharma Inc., hereby certify, to my knowledge, that:

1. the accompanying Quarterly Report on Form 10-Q of Benitec Biopharma Inc. for the three month period ended March 31, 2025 (the “Report”) fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities and Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of Benitec Biopharma Inc.

IN WITNESS WHEREOF, the undersigned has executed this Statement as of the date first written above.

/s/ Megan Boston

Megan Boston

Chief Financial Officer and Secretary (principal financial
and accounting officer)