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

# FORM 8-K

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): November 02, 2025**

# Benitec Biopharma Inc.

**(Exact name of Registrant as Specified in Its Charter)**

| **Delaware** | **001-39267** | **84-4620206** |
|---|---|---|
| **(State or Other Jurisdiction of Incorporation)** | **(Commission File Number)** | **(IRS Employer Identification No.)** |
| **3940 Trust Way Hayward, California** | | **94545** |
| **(Address of Principal Executive Offices)** | | **(Zip Code)** |

**Registrant's Telephone Number, Including Area Code: (510) 780-0819**

**(Former Name or Former Address, if Changed Since Last Report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**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 is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

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. ☐

**Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.**

On November 2, 2025, the Board of Directors (the "Board") of Benitec Biopharma Inc. (the "Company") appointed Dr. Sharon Mates to serve as a director of the Board, effective as of November 3, 2025. Dr. Mates was appointed as a Class I member of the Board with a term lasting until the Company's 2026 annual meeting of stockholders. The Board has determined that Dr. Mates is independent in accordance with applicable rules of the Nasdaq Stock Market LLC and the Company's Corporate Governance Guidelines.

In connection with Dr. Mates's appointment to the Board, the Board approved the grant of a options to purchase shares of the Company's common stock ("Common Stock") to Dr. Mates (the "Options") under the Company's 2020 Equity and Incentive Compensation Plan (the "Equity Plan"). The grant date of the Options will be November 5, 2025 (the "Grant Date"). The number of shares of Common Stock subject to the Options will be determined by dividing $450,000 by the closing price of the Common Stock as quoted on the Nasdaq on the Grant Date. The Options will vest in full on the earlier of immediately prior to the 2026 annual meeting of stockholders and the first anniversary of the Grant Date. The Options will be subject to the terms and conditions of the Equity Plan and the Company's standard form of stock option award agreement. Dr. Mates will also be entitled to the compensation provided to the Company's non-employee directors as described in the Company's Definitive Proxy Statement on Schedule 14A filed on October 14, 2025. Dr. Mates will also enter into the Company's standard indemnification agreement for members of the Board, the form of which is attached as Exhibit 10.6 to the Company's Annual Report on Form 10-K filed on September 22, 2025.

Other than the above Options grant, there have been no transactions, nor are there any currently proposed transactions, in which the Company was or is to be a participant and which Dr. Mates, or any member of her immediate family was or is to have a material interest, that would require disclosure under Item 404(a) of Regulation S-K.

The Company issued a press release announcing the appointment of Dr. Mates to the Board on November 3, 2025, a copy of which is attached hereto as Exhibit 99.1.

**Item 7.01 Regulation FD Disclosure.**

On November 3, 2025, Company issued a press release announcing that the FDA granted Fast Track Designation to BB-301 for the treatment of Oculopharyngeal Muscular Dystrophy ("OPMD"), a chronic, life-threatening genetic disorder. In addition, the press release announced that the Company would host a live webcast of the Company's latest interim clinical data presentation at 8:00 am (Eastern Time) on November 3, 2025. A copy of this press release is furnished as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated by reference herein to this Item 7.01. A copy of the presentation to be made in the webcast is attached hereto as Exhibit 99.3 to this Current Report on Form 8-K and is incorporated by reference herein to this Item 7.01.

The information included in this Current Report on Form 8-K (including Exhibits 99.2 and 99.3 hereto) that is furnished pursuant to this Item 7.01 shall not be deemed to be "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that Section or Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended. In addition, the information included in this Current Report on Form 8-K (including Exhibits 99.2 and 99.3 hereto) that is furnished pursuant to this Item 7.01 shall not be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing, unless expressly incorporated by specific reference into such filing.

**Item 8.01 Other Events.**

On November 3, 2025, the Company announced that the FDA granted Fast Track Designation to BB-301 for the treatment of OPMD, a chronic, life-threatening genetic disorder.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit No. | Description |
| --- | --- |
| 99.1 | Press Release of Benitec Biopharma Inc. (Board Appointment) dated November 3, 2025 |
| 99.2 | Press Release of Benitec Biopharma Inc. (Fast Track Designation) dated November 3, 2025 |
| 99.3 | Presentation of Benitec Biopharma Inc. |
| 104 | Cover Page Interactive Data File (embedded within the Inline XBRL document) |

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**BENITEC BIOPHARMA INC.**

Date: November 3, 2025

By: /s/ Dr. Jerel A. Banks
Dr. Jerel A. Banks
Chief Executive Officer

# Benitec Biopharma Announces Appointment of Sharon Mates Ph.D. to its Board of Directors

*-Dr. Mates served as the Chairman, Chief Executive Officer, and Co-founder of Intra-Cellular Therapies, a mental health company, from June 2002 until it was acquired by Johnson & Johnson in 2025-*

HAYWARD, Calif., November 3, 2025 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) ("Benitec" or "Company"), a clinical-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on its proprietary "Silence and Replace" DNA-directed RNA interference ("ddRNAi") platform, today announces the appointment of Dr. Sharon Mates to the board of directors (BOD) of the Company, effective November 2, 2025.

"We are delighted to welcome Dr. Sharon Mates to Benitec's Board of Directors," said Jerel Banks, M.D., Ph.D., Chairman and Chief Executive Officer of Benitec Biopharma**.** "Dr. Mates brings exceptional leadership experience in building successful biotechnology companies and advancing innovative therapies from discovery through commercialization. Her extensive track record of building value in the biotechnology sector will be invaluable as we continue progressing our BB-301 program in Oculopharyngeal Muscular Dystrophy (OPMD) towards regulatory approval and expanding the potential of our ddRNAi therapy platform into other therapeutic indications."

"I am pleased to join the Board of Directors at Benitec Biopharma during this critical period in the Company's evolution," said Dr. Sharon Mates**.** "Benitec's innovative silence and replace platform holds tremendous potential, and BB-301 represents the potential first of its kind gene therapy for addressing a serious unmet medical need. I look forward to working with the Board and the dedicated management team to contribute to Benitec's mission of delivering transformative treatments to patients while creating value for its shareholders."

Dr. Sharon Mates served as the Chairman, Chief Executive Officer, and Co-founder of Intra-Cellular Therapies Inc. (ITI) from June 2002 until its acquisition by Johnson & Johnson (JNJ). Intra-Cellular Therapies focused on developing medicines for mental health disorders like bipolar disorder, depression and schizophrenia and received U.S. Food and Drug Administration (FDA) approval for its novel antipsychotic CAPLYTA® in 2019 prior to its 2025 acquisition for approximately $14.6 billion by JNJ. She has served on several not-for-profit boards. Dr. Mates is also a director of Medincell, a biopharmaceutical company in France (Euronext: MEDCL). Dr. Mates received a B.S. from Ohio State University, a Ph.D. from the University of Washington and completed her postdoctoral fellowships at Massachusetts General Hospital and Harvard Medical School.

**About BB-301**

BB-301 is a novel, modified AAV9 capsid expressing a unique, single bifunctional construct promoting co-expression of both codon-optimized Poly-A Binding Protein Nuclear-1 (PABPN1) and two small inhibitory

RNAs (siRNAs) against mutant PABPN1. The two siRNAs are modeled into microRNA backbones to silence expression of faulty mutant PABPN1, while allowing expression of the codon-optimized PABPN1 to replace the mutant with a functional version of the protein. We believe the silence and replace mechanism of BB-301 is uniquely positioned for the treatment of OPMD by halting mutant expression while providing a functional replacement protein.

**About Benitec Biopharma, Inc.**

Benitec Biopharma Inc. ("Benitec" or the "Company") is a clinical-stage biotechnology company focused on the advancement of novel genetic medicines with headquarters in Hayward, California. 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. The Company is developing Silence and Replace-based therapeutics for chronic and life-threatening human conditions including Oculopharyngeal Muscular Dystrophy (OPMD).

A comprehensive overview of the Company can be found on Benitec's website at www.benitec.com.

**Forward Looking Statements**

Except for the historical information set forth herein, the matters set forth in this press release include forward-looking statements, including statements regarding Benitec's plans to develop and commercialize its product candidates and the clinical utility and potential attributes and benefits of ddRNAi and Benitec's product candidates, and other forward-looking statements.

These forward-looking statements are based on the Company's current expectations and subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: 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; 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 FDA and other governmental authorities and other regulatory developments; the Company's ability to protect and enforce its patents and other intellectual property rights; the Company's dependence on its relationships with its collaboration partners and other third parties; the efficacy or safety of the Company's products and the products of the Company's collaboration partners; the acceptance of the Company's products and the products of the Company's collaboration partners in the marketplace; 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, the impact of local, regional, and national and international economic conditions and events; and other risks detailed from time to time in the Company's reports filed

with the Securities and Exchange Commission. The Company disclaims any intent or obligation to update these forward-looking statements.

**Investor Relations Contact:**
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**Benitec Biopharma Provides Positive Interim Clinical Study Results for BB-301 Phase 1b/2a Clinical Trial and Receives FDA Fast Track Designation for BB-301**

- *Fast Track Designation was granted for BB-301 following FDA review of positive interim clinical study results and proprietary Responder Analysis planned for use in pivotal study for BB-301*
    - *BB-301 has also been granted Orphan Drug Designation from both FDA and EMA*

- *All six patients enrolled into Cohort 1 met the formal statistical criteria for response to BB-301, representing **a 100% response rate***
    - *Following the administration of BB-301, Cohort 1 patients experienced significant continuing reductions in dysphagic symptom burden, post-swallow residue accumulation, time required to consume fixed volumes of liquid, and improved pharyngeal closure during swallowing*

- *First patient in Cohort 2 successfully treated with BB-301 in fourth quarter of 2025*

- *Benitec plans to meet with the FDA in 2026 to confirm the BB-301 pivotal study design*

- *Dr. Sharon Mates, who served as Chairman, Chief Executive Officer, and Co-founder of Intra-Cellular Therapies Inc., appointed to the Benitec Biopharma Board of Directors as previously disclosed*

HAYWARD, Calif., November 3, 2025 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) ("Benitec" or "Company"), a clinical-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on its proprietary "Silence and Replace" DNA-directed RNA interference ("ddRNAi") platform, today provides positive interim clinical results for the BB-301 Phase 1b/2a Clinical Trial. Following administration of BB-301, Cohort 1 patients demonstrated significant and sustained improvements across multiple clinical measures including dysphagic symptom burden, post-swallow residue accumulation, time required to consume fixed volumes of liquid, as well as improved pharyngeal closure during swallowing. All six patients enrolled into Cohort 1 met the formal statistical criteria for response to BB-301, representing a 100% response rate. Following review of these encouraging interim data, the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to BB-301 for the treatment of OPMD with dysphagia. BB-301 was also previously granted Orphan Drug Designation from both the FDA and European Medical Association (EMA).

"Progressive dysphagia is a severe, life-threatening complication of OPMD which impacts 97% of OPMD patients, often leading to serious health risks, such as chronic choking, malnutrition, aspiration pneumonia, and death. We are excited by the profound effect that BB-301 can potentially have on this progressive disease as demonstrated by the interim clinical trial results

for Cohort 1, where 100% of patients were responders" said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec Biopharma Inc. "Securing Fast Track designation for BB-301 reflects the strength of our clinical data and the urgency of the unmet need in OPMD. This recognition validates our team's scientific and strategic execution, and we look forward to continued collaboration with the FDA as we advance toward a pivotal clinical trial."

The pre-treatment data for Cohort 1 patients reflect the first six months of Natural History Study follow-up and the final pre-treatment visit (i.e., the Phase 1 Screening Visit)

The interim post-treatment data for Cohort 1 patients reflect the following:

- 12-months of post-BB-301-treatment follow-up for Patient 1 and Patient 2
- 9-months of post-BB-301-treatment follow-up for Patient 3
- 6-months of post-BB-301-treatment follow-up for Patient 4 and Patient 5; and
- 3-months of post-BB-301-treatment follow-up for Patient 6

As the total dysphagic symptom burden experienced by OPMD patients has several known underlying contributors, the development of a multi-component composite endpoint to evaluate the potential treatment effects of BB-301 allows for incorporation of multiple discrete assessments that, in total, assess disease progression and treatment benefit of BB-301.

The BB-301 Responder Analysis (the multi-component composite endpoint) is comprised of a combination of patient-reported outcome results, objective assessment results, and swallowing capacity assessment results:

- Patient-Reported Outcome assessment results include: Sydney Swallow Questionnaire or **"SSQ"** results
- Objective Assessment Results include: Videofluoroscopic swallowing study results (Pharyngeal Area at Maximum Constriction or **"PhAMPC"**, Post-Swallow Pharyngeal Residue as measured by Total Pharyngeal Residue or **"TPR"** and Normalized Residue Ratio Scale or **"NRRS"**, Frequency of sequential swallows or **"SEQ"**)
- Functional Swallowing Capacity Assessment Results include: Clinically administered drinking assessment results (as measured by the cold-water timed drinking test or **"CWDT"**)

Following the administration of BB-301, Cohort 1 patients experienced clinically significant reductions, and met the formal statistical criteria for response, in the following assessments:

**Summary of Cohort 1 Results**

| | | PhAMPC | | TPR | | $NRRS_v$ | |
|---|---|---|---|---|---|---|---|
| | SSQ | Liquids | All Consistencies | Liquids | All Consistencies | Liquids | All Consistencies |
| Post-treatment Improvement | 328-point decline | 40% decline | 42% decline | 45% decline | 49% decline | 69% decline | 63% decline |

To date, the Benitec OPMD Natural History Study and the BB-301 Phase 1b/2a Clinical Trial represent the only clinical studies ever conducted which employ serial evaluation of the dysphagic symptom burden of OPMD patients and serial radiographic evaluation of the anatomical and functional elements of swallowing in OPMD patients at a frequency of approximately every 3-months. **Positive interim clinical study results demonstrate the significant and durable clinical benefit achieved by patients treated with BB-301.**

**Company Webcast Information:**

**Webcast title:** Interim BB-301 Phase 1b/2a Clinical Study Update

A live webcast of the interim clinical data presentation, will be held at 8:00 AM ET on Monday, November 3, 2025, and can be accessed by clicking here.

The event replay and corresponding slides will be placed on the News & Events tab on the Investor page of the Benitec website.

**About BB-301**
BB-301 is a novel, modified AAV9 capsid expressing a unique, single bifunctional construct promoting co-expression of both codon-optimized Poly-A Binding Protein Nuclear-1 (PABPN1) and two small inhibitory RNAs (siRNAs) against mutant PABPN1 (the causative gene for OPMD). The two siRNAs are modeled into microRNA backbones to silence expression of faulty mutant PABPN1, while allowing expression of the codon-optimized PABPN1 to replace the mutant with a functional version of the protein. We believe the silence and replace mechanism of BB-301 is uniquely positioned for the treatment of OPMD by halting mutant expression while providing a functional replacement protein. BB-301 has received Orphan Drug Designation from the EMA and Orphan Drug and Fast Track Designations from the FDA.

**About Benitec Biopharma, Inc.**
Benitec Biopharma Inc. (“Benitec” or the “Company”) is a clinical-stage biotechnology company focused on the advancement of novel genetic medicines with headquarters in Hayward, California. 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.

The Company is developing Silence and Replace-based therapeutics for chronic and life-threatening human conditions including Oculopharyngeal Muscular Dystrophy (OPMD). A comprehensive overview of the Company can be found on Benitec's website at www.benitec.com.

**Forward Looking Statements**
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**Investor Relations Contact:**

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BENITEC BIOPHARMA

silencing genes for life®

OCULOPHARYNGEAL MUSCULAR DYSTROPHY (OPMD)

# A Debilitating, Progressive Disease With No Approved Therapies

**Positive Clinical Data Supporting Advancement of BB-301 in OPMD**

WEBCAST: November 3, 2025

NASDAQ
BNTC

# Safe Harbor Statement

Except for the historical information set forth herein, the matters set forth in this presentation include forward-looking statements, including statements regarding Benitec's plans to develop and commercialize its product candidates, the timing of the completion of pre-clinical and clinical trials, the timing of the availability of data from our clinical trials, the timing and sufficiency of patient enrollment and dosing in clinical trials, the timing of expected regulatory filings, and potential clinical utility and potential attributes and benefits of ddRNAi and Benitec's product candidates, and other forward-looking statements.

These forward-looking statements are based on the Company's current expectations and patient to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: 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; 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 patients in clinical trials; determinations made by the FDA and other governmental authorities and other regulatory developments; the Company's ability to protect and enforce its patents and other intellectual property rights; the Company's dependence on its relationships with its collaboration partners and other third parties; the efficacy or safety of the Company's products and the products of the Company's collaboration partners; the acceptance of the Company's products and the products of the Company's collaboration partners in the marketplace; 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; the impact of local, regional, and national and international economic conditions and events; and other risks detailed from time to time in the Company's reports filed with the Securities and Exchange Commission. The Company disclaims any intent or obligation to update these forward-looking statements.

# Positive Interim Data and Regulatory Milestones Position BB-301 as the First Potential Disease-Modifying Genetic Medicine for OPMD-Related Dysphagia

## PHASE 1b/2a CLINICAL EFFICACY

### 100% RESPONDER RATE IN COHORT 1

- All six patients met formal statistical criteria for response in the proprietary OPMD responder analysis
- Patients experienced significant, clinically meaningful improvements in swallowing function and quality of life

### MEANINGFUL IMPROVEMENTS POST-BB-301 ADMINISTRATION ACROSS KEY ENDPOINTS

- Improved **patient reported outcome** measures over time
- Improved **throat closure** over time
- Improved **pharyngeal residue** over time
- Improved **functional swallowing capacity** over time

### FIRST PATIENT IN COHORT 2 SUCCESSFULLY TREATED

- Cohort 2 was initiated in 4Q2025

## SAFETY

### NO TREATMENT-RELATED SEVERE ADVERSE EVENTS

## REGULATORY

### FAST TRACK DESIGNATION (FDA)

Granted following review of positive interim clinical results and proprietary OPMD responder analysis

### ORPHAN DRUG DESIGNATION (FDA & EMA)

Recognizes BB-301's potential to address a rare, life-threatening disease with no approved therapies

**Benitec plans to meet with the FDA in 2026 to confirm all key elements of the anticipated BB-301 Pivotal Study design**

# Oculopharyngeal Muscular Dystrophy

## BB-301: A Novel Disease-Modifying Genetic Medicine

BENITEC BIOPHARMA

# Oculopharyngeal Muscular Dystrophy (OPMD) is a Debilitating Progressive Disease With No Approved Therapies

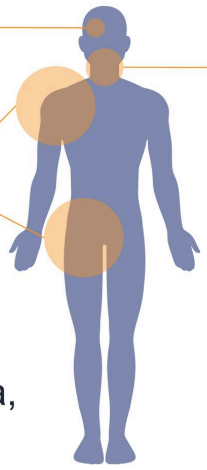
OPMD is a **rare autosomal-dominant degenerative muscle disorder**, caused by a mutation in the poly(A)-binding protein nuclear **1 (PABPN1) gene**

**PABPN1** is a ubiquitous protein that controls the length of mRNA poly(A) tails, mRNA export from the nucleus and alternative poly(A) site usage

**OPMD ONSET: TYPICAL AGE IS 40s-50s**

Eyelid drooping **(ptosis)** which can be asymmetric at onset

**Proximal limb weakness**



Difficulty swallowing **(dysphagia)** and choking during meals

**Progressive dysphagia impacts 97% of OPMD patients** and is a severe, life-threatening complication of OPMD which can lead to chronic choking, malnutrition, aspiration pneumonia and death

**~15K** Patients in North America, Europe and Israel

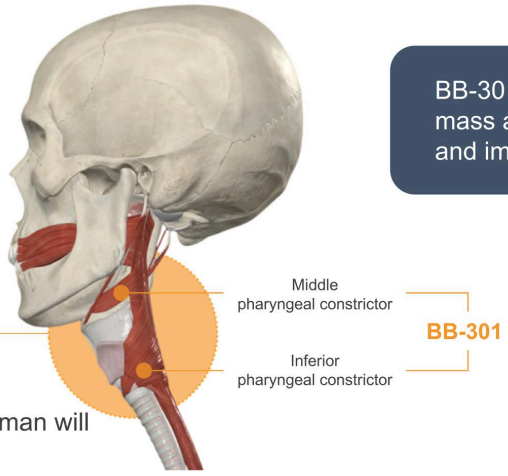
**BB-301 is the only clinical-stage therapeutic in development designed to treat dysphagia in patients with OPMD**

# Swallowing Overview and the Rationale for BB-301 Evaluation in Oculopharyngeal Muscular Dystrophy (OPMD)

OPMD weakens pharyngeal muscles, causing severe swallowing difficulties **(dysphagia)**

**Dysphagia** leads to choking, malnutrition, aspiration pneumonia[1]

Anatomical Structures of the Pharynx and BB-301 Injection Sites




BB-301 is designed to increase muscle mass and function, reducing dysphagia and improving patient outcomes

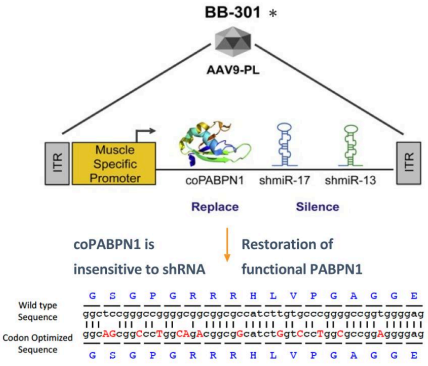
**BB-301** is delivered to the pharyngeal constrictor muscles via **direct intramuscular injection in the operating room**

**18-400** **times per hour** a healthy human will spontaneously swallow[2]

1. https://www.mayoclinic.org/diseases-conditions/dysphagia/symptoms-causes/syc-20372028 2. Rudney et al. (1995)

# BB-301: A Disease-Modifying Genetic Medicine for OPMD-Related Dysphagia

- **NOVEL "SILENCE AND REPLACE" PLATFORM** BB-301 uses DNA-directed RNA interference (ddRNAi) to simultaneously **silence the mutant gene** causing OPMD and **deliver a healthy, functional gene,** potentially providing a permanent solution with a single administration
- **ADDRESSES UNMET NEEDS** The ddRNAi approach can tackle diseases that are not treatable by gene silencing or gene therapy alone
- **TARGETED GENE THERAPY** BB-301 is designed to block production of the harmful PABPN1 protein and restore normal muscle function by supplying a new, functional version of the protein
- **PRECISE DELIVERY** Administered directly into the pharyngeal constrictor muscles via intramuscular injection in the operating room, BB-301 maximizes local benefit and minimizes systemic exposure.
- **CLINICAL DOSING** Evaluated at doses of 1.2e13 vg/patient (Cohort 1) and 1.8e13 vg/patient (Cohort 2)

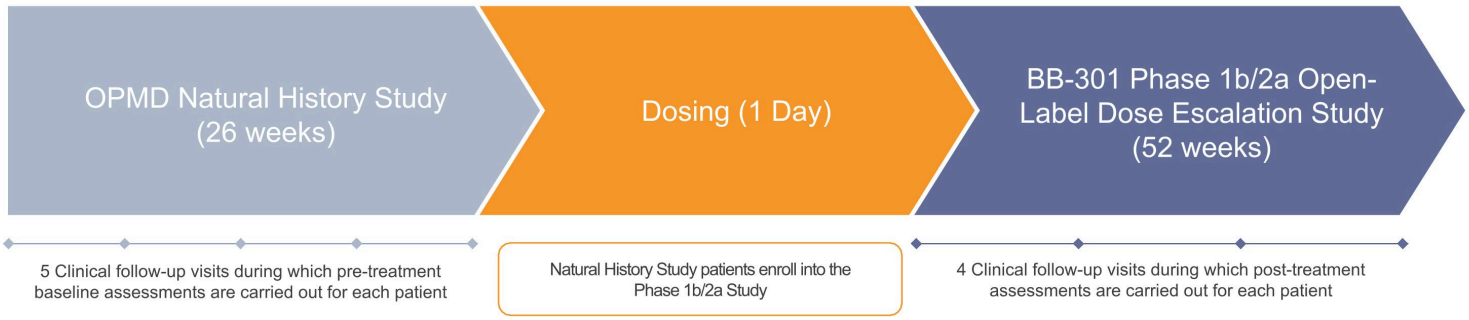



* Strings-Ufombah, et al., Molecular Therapy: Nucleic Acids, Vol. 24, 67-78, June 2021

# Study Design and Key Efficacy Endpoints of the OPMD Natural History Study and BB-301 Phase 1b/2a Clinical Study

BENITEC BIOPHARMA

# BB-301: Clinical Development Program




- 23 patients have been enrolled in the OPMD Natural History Study to date
  - Natural History Study patients may be eligible for the pivotal study
- All patients are blinded to their Total SSQ Scores and VFSS (TPR and Sequential Swallowing) assessment results, and the Central Reader for the VFSS assessments is blinded to the SSQ Scores for all patients
- The BB-301 Phase 1b/2a Open-Label Dose Escalation Study can enroll up to 30 patients across 3 doses
  - **All 6 Cohort 1 patients have been treated with BB-301**
  - **The first Cohort 2 patient has been successfully treated with BB-301**
  - **Primary endpoint is safety, secondary endpoints include a range of efficacy assessments**

# BB-301: Serial Characterization of Dysphagia Severity Informs Efficacy Assessment

- The Natural History Study and BB-301 Phase 1b/2a Clinical Study include comprehensive assessments of dysphagia approximately every 3 months
- The total dysphagic symptom burden experienced by OPMD patients has several known underlying contributors
- The serial assessments of dysphagia facilitated the creation of a multi-component responder analysis which incorporates multiple discrete assessments that holistically assess disease progression and treatment benefit of BB-301

**Efficacy assessments were derived from literature-based methods used to assess OPMD dysphagic symptom burden and include patient-reported outcomes, objective anatomic assessments, and functional parameters**

Patient-Reported Oral-Pharyngeal Dysphagia via use of a **clinically validated** 17-question patient reported outcome instrument, Sydney Swallow Questionnaire **(SSQ)**

Videofluoroscopic Swallowing Studies **(VFSS)** of liquids and solids for anatomic and functional assessments of:

- Pharyngeal constrictor muscle function via assessment of Pharyngeal Area at Maximum Pharyngeal Constriction **(PhAMPC)**
- Swallowing Efficiency via assessment of post-swallow accumulation of food/liquid material
  - Total Pharyngeal Residue **(TPR)**
  - Normalized Residue Ratio Scale, Valleculae **($NRRS_v$)**
- Swallowing Effectiveness via assessment of frequency of pathologic sequential swallows **(SEQ)**

Functional swallowing capacity as measured by the Cold Water Timed Drinking Test **(CWDT)**

# BB-301: Cohort 1 Pre-Treatment Trends and Post-Treatment Trends

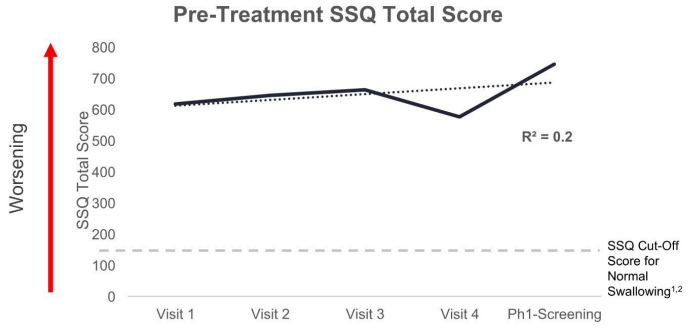
- The **pre-treatment period** includes the first six months of natural history study follow-up and the screening visit for enrollment in the Phase 1b/2a study
- The interim **post-treatment period** includes all clinical assessments accrued to date for Cohort 1 patients:
  - 12-months of post-BB-301-treatment follow-up for Patient 1 and Patient 2
  - 9-months of post-BB-301-treatment follow-up for Patient 3
  - 6-months of post-BB-301-treatment follow-up for Patient 4 and Patient 5
  - 3-months of post-BB-301-treatment follow-up for Patient 6
- Swallowing tasks comprised liquids (thin, moderately thick and extremely thick) and solid food

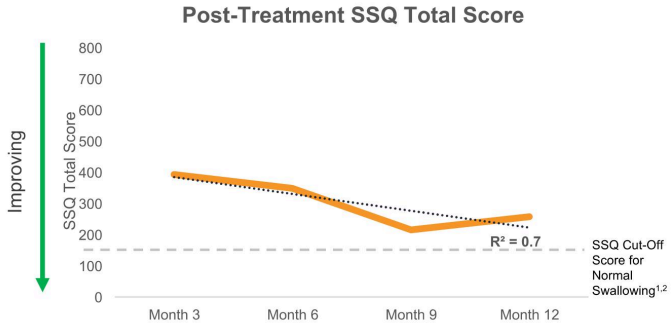
**The following assessments met the statistical requirement for response:**

| | | PhAMPC | | TPR | | $NRRS_v$ | |
|---|---|---|---|---|---|---|---|
| | SSQ | Liquids | All Consistencies | Liquids | All Consistencies | Liquids | All Consistencies |
| **Post-treatment Improvement** | 328-point decline | 40% decline | 42% decline | 45% decline | 49% decline | 69% decline | 63% decline |

# BB-301: Cohort 1 Demonstrated a Highly Clinically Meaningful Improvement in Sydney Swallow Questionnaire (SSQ) Total Score

- Pre-Treatment = 586
- Post-Treatment = 258
- **328-point decline in SSQ Total Score post-BB-301 treatment**

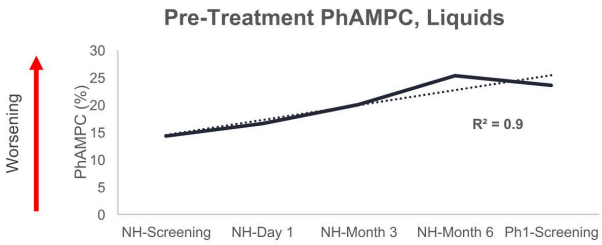


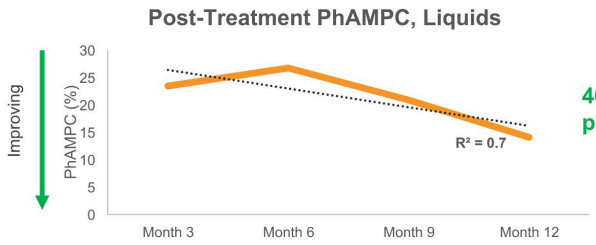




**Total SSQ Score Improves Over Time Following BB-301 Administration and the Statistical Requirement for Improvement Has Been Achieved**

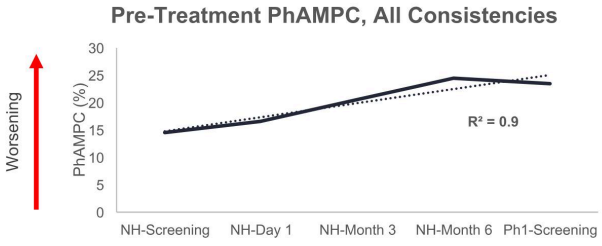
1. Bua, B.A. and Bülow, M., BMC Research Notes (2014) 7:742, 2. Audag N., et al., Dysphagia (2019) 34:556-566

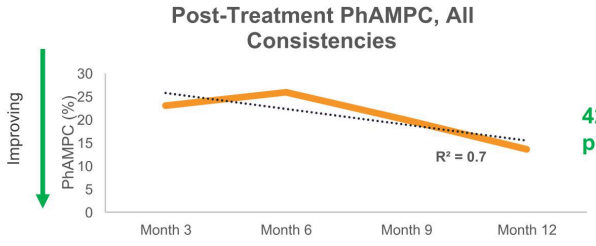
# BB-301: Cohort 1 Demonstrated a Highly Clinically Meaningful Improvement in Pharyngeal Area at Maximum Pharyngeal Constriction (PhAMPC)




**40% decline in PhAMPC post-BB-301 treatment**

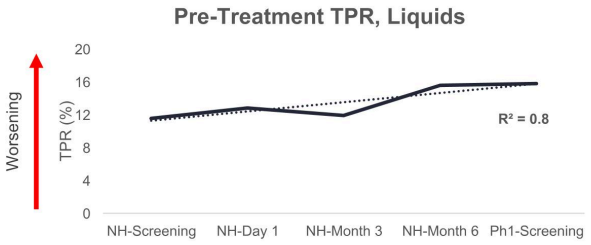


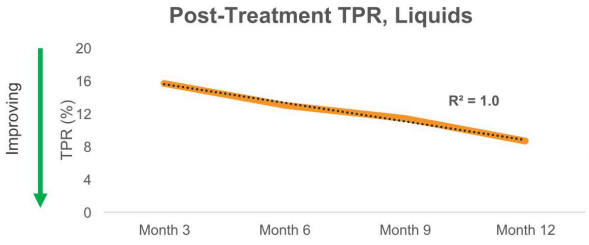




**42% decline in PhAMPC post-BB-301 treatment**

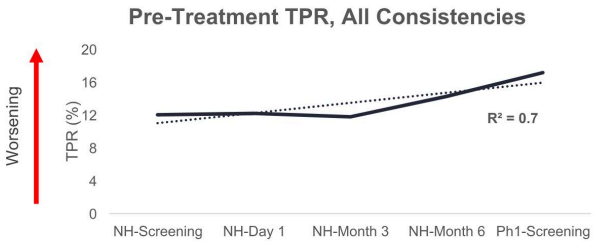
**PhAMPC Improves Over Time Following BB-301 Administration and the Statistical Requirement for Improvement Has Been Achieved**

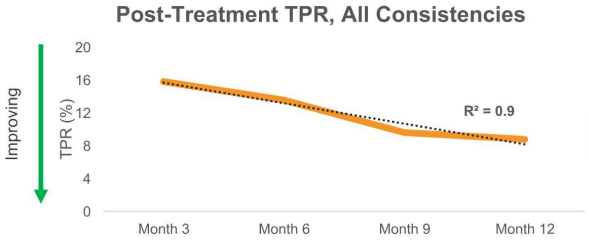
# BB-301: Cohort 1 Demonstrated a Highly Clinically Meaningful Improvement in Total Pharyngeal Residue (TPR)




**45% decline in TPR post-BB-301 treatment**

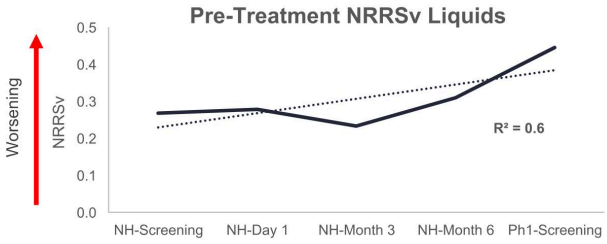


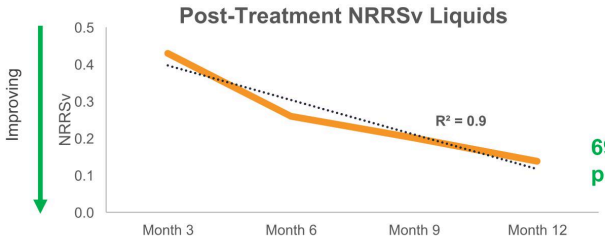




**49% decline in TPR post-BB-301 treatment**

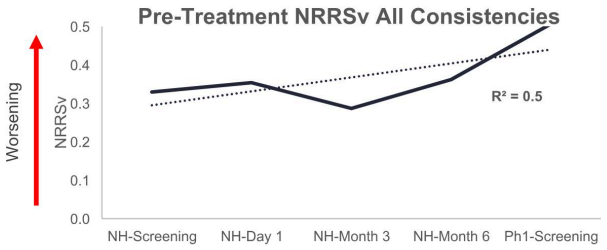
**TPR Improves Over Time Following BB-301 Administration and the Statistical Requirement for Improvement Has Been Achieved**

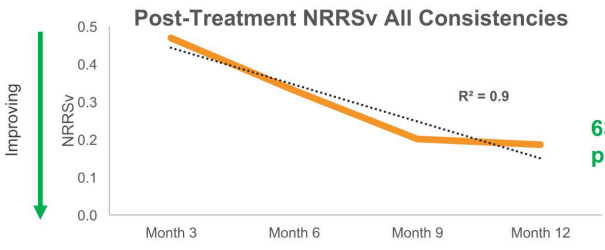
# BB-301: Cohort 1 Demonstrated a Highly Clinically Meaningful Improvement in Normalized Residue Ratio Scale for the Valleculae (NRRSv)




**69% decline in NRRSv post-BB-301 treatment**



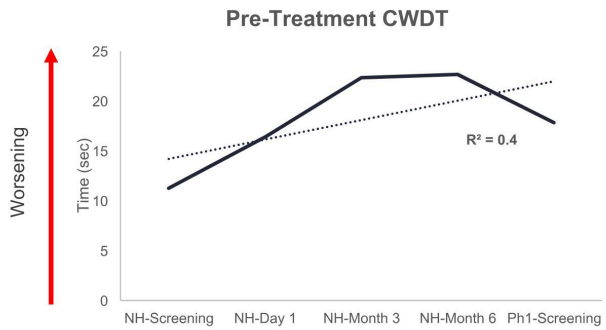



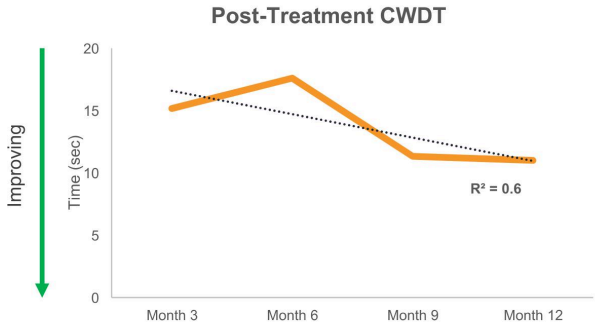

**63% decline in NRRSv post-BB-301 treatment**

**NRRSv Improves Over Time Following BB-301 Administration and the Statistical Requirement for Improvement Has Been Achieved**

# BB-301: Cohort 1 Demonstrated a Clinically Meaningful Improvement in Cold Water Timed Drinking Test (CWDT)

- Pre-Treatment = 18.1 seconds
- Post-Treatment = 11.0 seconds
- **Post-treatment scores were reduced as compared to the pre-treatment period**




**CWDT Improves Over Time Following BB-301 Administration, However, at the Time of this Interim Analysis, Statistical Criteria for Improvement Have Not Yet Been Met**

# BB-301: Natural History Trends of Cohort 1 Patients Inform Potential Pivotal Study Design and the Development of a Proprietary Responder Analysis

- Without intervention, the functional elements of the swallowing process worsen over time for OPMD patients, as shown in the pre-treatment results for Cohort 1 patients.
- The progressive loss of swallowing function drives the clinically significant symptom burden experienced by the OPMD patients.
- Pre-treatment, Cohort 1 patients exhibited worsening pharyngeal residue post-swallow (TPR and NRRSv), worsening pharyngeal area at maximum pharyngeal constriction (PhAMPC), abnormal total SSQ scores, and abnormal CWDT scores.

- Each patient enrolled into the BB-301 clinical development program serves as their own control.
  - **This approach was recently highlighted by the FDA in the Sept 2025 Draft Guidance for Industry entitled "Innovative Designs for Clinical Trials of Cellular and Gene Therapy Products in Small Populations"**
- Evidence of stabilization or improvement, as seen post-BB-301 treatment for Cohort 1 patients, represents clinically meaningful improvement.
  - Post BB-301 treatment, TPR, NRRSv, PhAMPC and SSQ results improved significantly.
  - Post-treatment CWDT scores were reduced as compared to those observed in the pre-treatment period, however, at the time of this interim analysis the statistical criteria for the formal designation of improvement have not yet been met.

# BB-301 Responder Analyses: A Framework for Evaluation of Efficacy

- The total dysphagic symptom burden experienced by OPMD patients has several known underlying contributors
- Development of a multi-component composite endpoint to evaluate the potential treatment effects of BB-301 allows for incorporation of multiple discrete assessments that, in total, assess disease progression and treatment benefit of BB-301
- The Responder Analysis is comprised of a combination of patient-reported outcome results, objective assessment results, and swallowing capacity assessment results:
  - Patient-Reported Outcome Assessment results include: Sydney Swallow Questionnaire **(SSQ)** results
  - Objective Assessment Results include: Videofluoroscopic swallowing study results (Pharyngeal Area at Maximum Pharyngeal Constriction **[PhAMPC]**, Post-Swallow Pharyngeal Residue [Total Pharyngeal Residue or **"TPR"** and Normalized Residue Ratio Scale or **"NRRS"**], Frequency of sequential swallows **"SEQ")**
  - Functional Swallowing Capacity Assessment Results include: Clinically administered drinking assessment results (as measured by the cold-water timed drinking test **"CWDT"**)
- Results are combined into a single framework that facilitates the overall assessment of the clinical benefit achieved by each study patient.

**Interim clinical results using this analysis were submitted to the FDA over the course of an interactive process that supported the grant of Fast Track Designation for BB-301**

# Proprietary OPMD Responder Analysis Description

| Assessment | Requirement for a Responder | Responder Scoring |
|---|---|---|
| Patient-Reported Oral-Pharyngeal Dysphagia determined by **SSQ** Total Score | SSQ must not worsen over the post-treatment period ***(if this criteria is not met, then no further analyses are done; the patient is not a responder).*** | Maximum score of 1 for this component if requirement is met |
| Pharyngeal Constrictor Muscle Function as measured by **PhAMPC** | A patient is considered a responder if statistical criteria* are achieved for at least 1 out of the 4 consistencies evaluated | Maximum score of 1 for this component if PhAMPC for at least 1 consistency assessment meets responder criteria |
| Frequency of Pathologic Sequential Swallows (**SEQ**): Number of sequential swallows (detected during VFSS as a series of involuntary contractions of the pharyngeal muscles without restoration of the resting pharyngeal diameter between contractions) | A patient is considered a responder if they experience a 30% decrease in the total frequency of sequential swallows post-treatment compared to pre-treatment | Maximum score of 1 for this component if the total frequency of sequential swallows decreases by ≥ 30% post-treatment with BB-301 |
| Swallowing Efficiency as measured by either **NRRSv** or **TPR** | A patient is considered a responder if statistical criteria* are achieved for at least 1 out of the 8 consistency assessments evaluated | Maximum score of 1 for this component if at least 1 consistency assessment meets responder criteria |
| Functional Swallowing Capacity as measured by the **CWDT** | A patient is considered a responder if statistical criteria* are achieved | Maximum score of 1 for this component if requirement is met |

**A maximum total score of 5 "Yes" values can be achieved if all criteria above are met.**
**If a score of ≥ 2 "Yes" values (≥ 40%) is achieved, then the patient is a Responder.**

*Formal statistical criteria were submitted to FDA over the course of the interactive process that supported the grant of Fast Track Designation for BB-301

# BB-301: 100% of Patients in Cohort 1 (n = 6) Were Responders

| Patient | Follow-Up at the time of Fast Track Submission | Response Criteria Achieved | Total Responder Score | Updated Follow-Up post Fast Track Submission* | Updated Response Criteria Achieved* | Updated Total Responder Score* | Responder? |
|---|---|---|---|---|---|---|---|
| Patient 1 | 12- months | SSQ, PhAMPC, Pharyngeal residue, CWDT | 4 | | | | ✓ |
| Patient 2 | 12- months | SSQ, PhAMPC, SEQ, CWDT | 4 | | | | ✓ |
| Patient 3 | 9- months | SSQ, SEQ, Pharyngeal residue | 3 | 12- months | SSQ, PhAMPC, SEQ, Pharyngeal residue | 4 | ✓ |
| Patient 4 | 6- months | SSQ, Pharyngeal residue | 2 | 9- months | SSQ, PhAMPC, Pharyngeal residue | 3 | ✓ |
| Patient 5 | 6- months | SSQ, Pharyngeal residue | 2 | | | | ✓ |
| Patient 6 | 3-months | SSQ, Pharyngeal residue | 2 | | | | ✓ |

Total Responder Score Increases Over Time

*Data pending source data verification



BENITEC BIOPHARMA

# BB-301: Transformative Potential for Patients with Oculopharyngeal Muscular Dystrophy (OPMD)

## ALL SIX COHORT 1 PATIENTS

Distinct causes of their respective total dysphagic symptom burdens were safely treated with BB-301

Experienced significant, clinically meaningful improvements in swallowing function and quality of life

## COHORT 2

First patient in Cohort 2 successfully treated with BB-301

## REGULATORY MILESTONES

Fast Track Designation was granted by the FDA

FDA and EMA Orphan Drug Designation

The company plans to engage the FDA in 2026 to confirm details of the planned OPMD pivotal trial

**Patients experienced significant reductions in their total dysphagic symptom burdens as assessed by the proprietary OPMD responder analysis**

**There have been no deaths and no treatment-related Severe Adverse Events for any patient enrolled in Cohort 1 to date**

**100% of patients in Cohort 1 (including those who have completed the statistical follow-up period as well as those with only interim clinical results) were determined to be responders to BB-301 treatment**

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# Appendix

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# BB-301: Potential One-Time Treatment Designed to Address the Root Cause of Oculopharyngeal Muscular Dystrophy (OPMD)-Related Dysphagia

| | |
|---|---|
| **High Unmet Need and Significant Market Opportunity** | • **There are no therapeutic interventions approved for OPMD**<br>• BB-301 is the only clinical-stage therapeutic agent in development designed to treat dysphagia (loss of the ability to swallow) in patients with OPMD<br>• 15,000 patients in the U.S., Europe, Canada, and Israel |
| **OPMD-Related Dysphagia Natural History Study Data Informs Putative Pivotal Study Design and a Potential Proprietary Responder Analysis** | • Progressive dysphagia impacts **97%** of OPMD patients and is a severe, life-threatening complication of OPMD which can lead to chronic choking, malnutrition, aspiration pneumonia, and death<br>• **Without intervention, the functional elements of the swallowing process worsened over time for OPMD patients prior to their enrollment into Cohort 1 of the BB-301 Phase 1b/2a Clinical Treatment Study**<br>• **The Natural History Study results facilitated the development of a putative proprietary Responder Analysis and potential pivotal study design, both of which were presented to the FDA in support of Fast Track Designation for BB-301** |
| **Transformative BB-301 Phase 1b/2a Clinical Study Results** | • BB-301 is delivered via direct intramuscular (IM) injection to pharyngeal constrictor muscles resulting in minimal systemic exposure; 6 patients have been safely treated with the low dose of BB-301 and there have been no treatment related severe adverse events<br>• Following the administration of BB-301, Cohort 1 patients experienced significant reductions in dysphagic symptom burden, reductions in post-swallow residue accumulation, reductions in the time required to consume fixed volumes of liquid, and improvements in pharyngeal closure during swallowing<br>• **All 6 patients enrolled into Cohort 1 met the formal statistical criteria for Response to BB-301, representing a 100% response rate** |

**Benitec continues to plan for a formal meeting with FDA in 2026 to confirm all key elements of the anticipated BB-301 Pivotal Study design**

# Robust, Blinded Methods are Utilized to Evaluate the Dysphagic Symptom Burden of each Study patient

- Patient-Reported Outcome Assessments: The SSQ is employed to serially evaluate the severity of dysphagia as reported by the patient at each clinical site visit
- X-ray Based Swallowing Assessments: VFSS are employed to serially analyze Swallowing Efficiency and Swallowing Effectiveness at each clinical site visit, with imaging results reviewed and rated via a standardized process

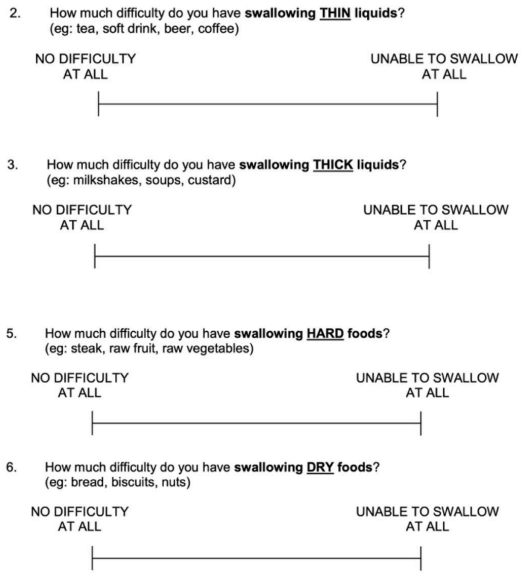
- Swallowing is an involuntary process: The standardized swallowing tasks employed during the VFSS are not impacted by the effort of the patient, as the autonomic nervous system controls the involuntary muscle movements comprising the pharyngeal phase of swallowing
- Blinded assessments: All Study patients are blinded to their Total SSQ Scores and VFSS Assessment Results, and the Central Reader for the VFSS assessments is blinded to the SSQ Scores for all Study patients

# Patient-Reported Oral-Pharyngeal Dysphagia: As Assessed by the SSQ

## Patient-reported oral-pharyngeal dysphagia as assessed by the SSQ:

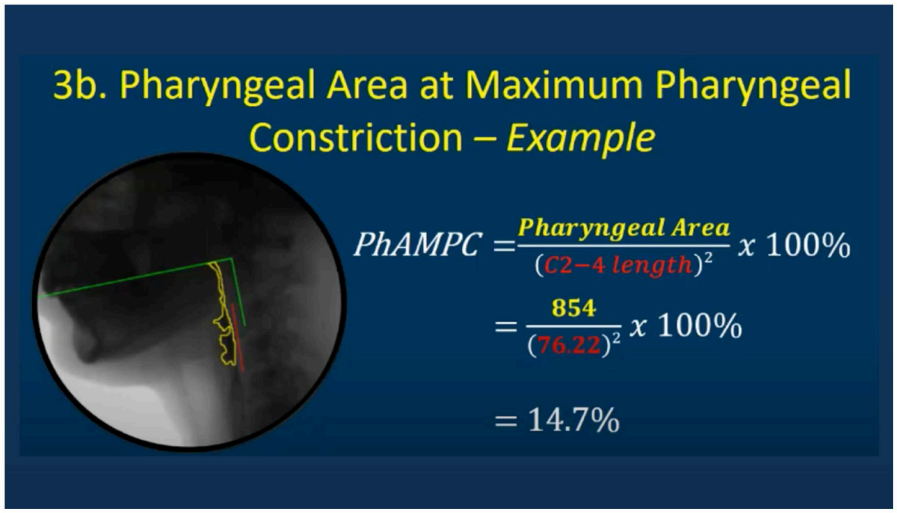
- The SSQ is a 17-item self-report inventory assessing subjective symptoms of oral-pharyngeal dysphagia
  - The questionnaire uses a 100-mm long visual analogue scale for all but 1 question
  - Possible scores range from 0 to 1700, with higher scores indicating greater swallowing difficulty
- The SSQ has demonstrated strong content, construct, discriminant, and predictive validity and test-retest reliability in a range of patient populations
- The SSQ evaluates a range of domains, including: difficulty swallowing specific food types/consistencies, frequency of choking during ingestion of specific food types/consistencies, and the requirement for multiple swallows during ingestion of food and liquid

## SSQ Example Questions




Szczesniak, M., et al, Dysphagia, 2014

# An Estimate of Pharyngeal Constrictor Muscle Function: As Measured by PhAMPC

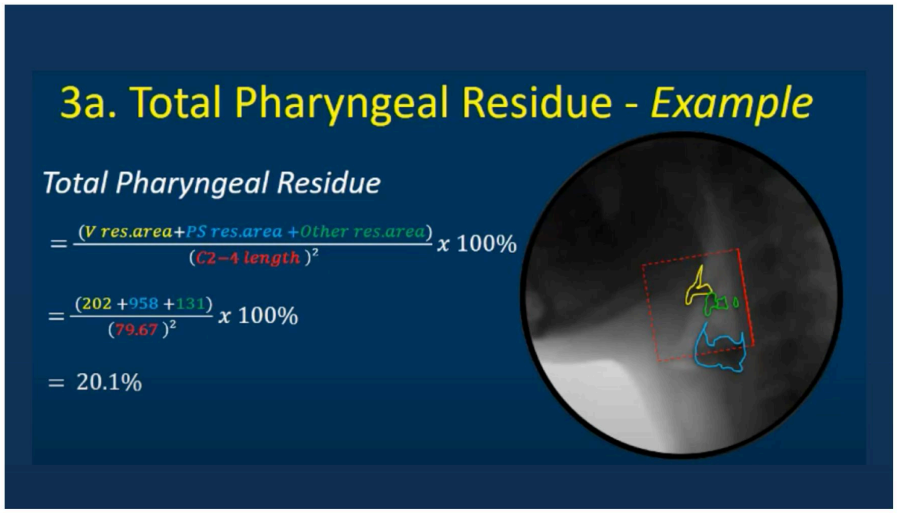



C2-C4 length serves as an anatomical scalar

- PhAMPC characterizes the area of the pharynx at the point of maximum constriction during swallowing
- Measurement (yellow) occurs on the videofluoroscopy frame with the smallest amount of unobliterated air space and barium-containing bolus visible in the pharynx
- Normal PhAMPC values span the range of 0% to 2.2% for the characteristic food and liquids that are evaluated in the swallowing tasks in the ongoing clinical studies

Steele, C., et al. ASHA 2019 Convention Session on the ASPEKT C Method of Videofluoroscopy Analysis

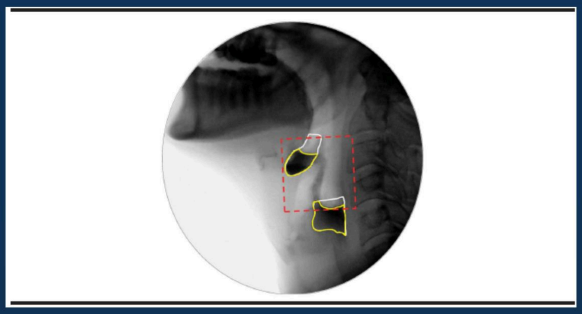
# Swallowing Inefficiency: As Measured by Post-Swallow TPR




C2-C4 length squared serves as an anatomical scalar

- Total Pharyngeal Residue measurement comprises the amount of food or liquid material remaining in the pharynx after the first swallow of the bolus
- Normal Total Pharyngeal Residue values should be close to zero

# Swallowing Inefficiency: As Measured by NRRSv, a Measure of Vallecular Residue



- Valleculae are the bilateral spaces between the base of the tongue and the epiglottis

C2-C4 length squared (dashed red line) serves as an anatomical scalar and spatial housing areas are shown in white[2]
Measurement of residue (yellow) occurs on the first frame showing pyriform sinuses at lowest position[2]

$$NRRS = \left(\frac{Residue\ Area}{Housing\ Area}\right) \times 10 \times \left(\frac{Residue\ Area}{Scalar^2}\right)$$

- NRRS incorporates the ratio of residue occupying a pharyngeal space and a proportion expressing the area of observed residue normalized against the size of an individual[1]
- Pharyngeal residue constitutes a risk for post-swallow aspiration, with clinically significant residue determined by NRRSv values ≥ 0.06[3]
- Residue accumulation in the valleculae (NRRSv ≥ 0.09) has also shown to be correlated with an increased risk of penetration-aspiration
- In a 2021 Dutch OPMD Natural History Study of 43-patients, a statistically significant decline in swallowing efficiency was captured via the use of longitudinal NRRSv assessments (2 assessments, occurring 20-months apart). The amount of abnormal pharyngeal residue in the valleculae increased significantly (mean NRRSv ratio 0.24 vs 0.13, p = 0.007) over this time period.[4]

1. Pearson W.G. et. al., Dysphagia, 2013; 2. Steele, C.M. et. al., Journal of Speech, Language and Hearing Research, 2019; 3. Molfenter S.M. et. al., Dysphagia, 2013; 4. Kroon, R.H.M.J.M. et. al., Neurology, 2021

# Swallowing Ineffectiveness: As Measured by the Frequency of Pathologic Sequential Swallows (SEQ)

Sequential swallowing is defined as the completion of two or more consecutive swallows in rapid succession where the hyolaryngeal complex (HLC) does not return to rest and the pharynx lacks complete patency between swallows[1]

- Under normal physiologic conditions, sequential swallowing occurs during continuous drinking of large volumes **(90 mL or greater)** of thin liquids via straw, cup, or bottle (for reference, one can of a soft drink has a volume of approximately 355 mL of thin liquid)

For the BB-301 Phase 1b/2a Study, the return of the HLC to its resting position (i.e., achievement of pharyngeal muscle relaxation) between swallows is characterized on VFSS via the identification of the the "Swallow Rest Frame"

- Swallow Rest is the terminal event of each discrete swallow, and the Swallow Rest Frame is identified as the first VFSS frame showing the pyriform sinuses at their lowest position, relative to the spine, prior to any hyoid burst or laryngeal elevation for a subsequent subswallow[2]

In dysphagic disorders (e.g., OPMD), during the consumption of small volumes of liquids **(e.g., less than 15 mL, as employed in the BB-301 Phase 1b/2a Study), pharyngeal muscle relaxation may not be achieved consistently between swallows (i.e., the HLC does not return to rest and the pharynx lacks complete patency);** instead, a patient may experience pathologic sequential swallows when consuming small volumes of liquids

**Pathologic sequential swallows occur during the consumption of small volumes of liquid and are detected during VFSS as a series of involuntary contractions of the pharyngeal muscles without restoration of the resting pharyngeal diameter between contractions**

1. Ambrocio, K. R., et al., Dysphagia (2023) 38:1497–1510; 2. Steele, C., et al., Journal of Speech, Language, and Hearing Research, Vol. 62, 1338–1363, May 2019

# Functional Swallowing Capacity: As Measured by the CWDT

- The CWDT measures the time required for a patient to swallow 80 mL of ice-cold water[1]
- The CWDT (dysphagia documented by the inability to swallow 80 mL of cold water in less than 8 seconds), alongside family history of the disease and the presence of at least one ptotic eyelid with a fissure of less than 8 mm, can be used to make a clinical diagnosis of OPMD[2]
  - Failure to drink 80 mL of water in less than 8 seconds suggests the presence of dysphagia in OPMD individuals (sensitivity = 87% and specificity = 77%)[3]
- The timed swallowing of 80 mL of cold water represents a global functional evaluation of swallowing and has been used as one of the endpoints to assess the outcome of an autologous myoblast transplant Phase 1/2a clinical study in OPMD patients[4]

1. Nathardwarawala, K.M. et. al. Journal of Neurology, Neurosurgery, and Psychiatry, 1992; 2. Codère, F. et. al., Orbit, 2001; 3. Côté, C. et. al., Dysphagia, 2024; 4. Périé, S. et. al., Molecular Therapy, 2013