November 14, 2024

Benitec Biopharma Releases First Quarter 2025 Financial Results and Provides Operational Update

-Positive 270-day Interim Clinical Study Data for the First Subject and Positive 180-day Interim Clinical Study Data for the Second Subject Treated with the Low-Dose of BB-301 in the Phase 1b/2a Clinical Treatment Study Reported in October as a Late-Breaking Oral Presentation at the 29th Annual Congress of the World Muscle Society-

-Third Subject Safely Treated with the Low-Dose of BB-301 in October 2024, and Fourth Subject Expected to Receive Treatment with the Low-Dose of BB-301 in December 2024

HAYWARD, Calif., Nov. 14, 2024 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) ("Benitec" or the "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 announced financial results for its first fiscal quarter ended September 30, 2024. The Company has filed its quarterly report on Form 10-Q for the quarter ended September 30, 2024 with the U.S. Securities and Exchange Commission (SEC).

"We recently announced the 270-day interim clinical study results for the first Subject and the 180-day interim clinical study results for the second Subject enrolled into the low-dose cohort of the BB-301 Phase 1b/2a Clinical Treatment Study. We continue to be extremely grateful for the strong support of the Subjects and their families and for their continued participation in the BB-301 clinical development program. We were highly encouraged by the significant, clinically meaningful improvements observed for both Subjects treated at the low-dose of BB-301, with Subject 1 experiencing durable improvements in core dysphagic symptoms of 35% to 40%, and Subject 2 achieving a clinically normal swallowing profile based on the results of the Sydney Swallow Questionnaire," said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec. "The third Subject was treated with the low-dose of BB-301 in October 2024, and we anticipate the treatment of the fourth Subject in December 2024. We remain optimistic about the potential for continued benefit in Subjects enrolled in the ongoing study and, including funds from recent exercises of shareholder-held warrants, Benitec is well-funded to advance the BB-301 clinical development program. We look forward to enrolling additional Subjects at the next, higher dose of BB-301, in 2025."

Operational Updates

The key milestones related to the development of BB-301 for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD)-related Dysphagia, are outlined below:

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

- At the lowest-dose of gene therapy BB-301 (1.2e13 vg/subject), there were no Significant Adverse Events observed.
- Dysphagic symptoms at baseline for Subject 1 (7-years post diagnosis) were more severe than those of Subject 2 (6-years post diagnosis) as assessed by pre-dose Sydney Swallow Questionnaire (SSQ) and Total Pharyngeal Residue (TPR) results, but both subjects experienced significant clinical benefit per the post-dose SSQ scores and TPR results.
- The SSQ Total Scores and SSQ Sub-Scores correlate strongly with the Videofluoroscopic Swallowing Study (VFSS) TPR results.
- Subject 1 experienced clinically meaningful improvements in post-dose SSQ Total Score and SSQ Sub-Scores at Day 270 driven by corresponding reductions in VFSS TPR values.
- Subject 2 experienced clinically meaningful improvements in post-dose SSQ Total Score and SSQ Sub-Scores at Day 180, with an SSQ Total Score representative of a normal swallowing profile, driven by a corresponding reduction in the frequency of pathologic low-volume sequential swallows.
- These data represent the first reported successful improvements in swallowing function following the use of a novel gene therapy for OPMD.

Subjects Enrolled in the BB-301 Clinical Development Program are Impacted by Two Discrete Drivers of Dysphagic Symptoms:

- Excessive accumulation of pharyngeal residue post-swallow (i.e., inefficiency of swallowing) represents one key driver of dysphagic symptoms.

- Pathologic low-volume sequential swallows represent a second key driver of dysphagic symptoms.

    - Pathologic low-volume sequential swallows are characterized on videofluoroscopic swallowing study examination as rapid contractions of the pharyngeal muscles without full restoration of the resting pharyngeal diameter between contractions.
    - This pharyngeal contraction pattern is aberrant and is not observed in healthy subjects during the consumption of low-volumes of thin liquids (e.g., 15 milliliters water).

Interim Clinical Study Results for Subject 1 (270-Days Post Treatment with BB-301):

- Global inefficiency of swallowing for solid food, thin liquid, and thick liquids drives dysphagia for Subject 1.
- Subject 1 displayed continued clinically meaningful reductions (i.e., improvements) in SSQ Total Score (35% reduction) and SSQ Sub-Scores (42% reduction for Thin Liquid, 16% reduction for Solid Food, and 22% reduction for Thick Liquids). Subject 1 displayed correspondingly significant reductions (i.e., improvements) in VFSS TPR (33% reduction for Thin Liquid, 18% reduction for Solid Food, and 30% 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-dose period.

Interim Clinical Study Results for Subject 2 (180-Days Post Treatment with BB-301):

- Pathologic low-volume sequential swallowing for thin liquid drives dysphagia for Subject 2. Pathologic low-volume sequential swallows are experienced by the Subject as multiple swallows and are detected during VFSS as a continuous series of rapid contractions of the pharyngeal muscles without full restoration of the resting pharyngeal diameter between contractions. The pathologic pattern of contractions interrupts the discrete peristaltic contractions typically observed during swallows of low volumes of thin liquids (e.g., 15 milliliters water), during which the resting diameter of the pharynx is fully restored between successive swallows.
- Subject 2 displayed clinically meaningful reductions (i.e., improvements) in SSQ Total Score (89% reduction) and the SSQ Sub-Score for the necessity of repeat swallows during consumption (84% reduction) as compared to the average values recorded for Subject 2 during the pre-dose period. The average post-dose SSQ Total Score of 82 is representative of a clinically normal swallowing profile for Subject 2. Subject 2 displayed correspondingly significant reductions (i.e., improvements) in the post-dose frequency of pathologic low-volume sequential swallows as evaluated by VFSS (92% reduction) following the administration of the low-dose of BB-301 as compared to the pre-dose values recorded for Subject 2.

Both Subjects were blinded to their SSQ Total Scores and VFSS TPR assessment results, and the Central Reader for the VFSS assessments was blinded to the SSQ Total Scores and SSQ Sub-Scores for each Subject.

Enrollment into the BB-301 Phase 1b/2a Clinical Treatment Study is Ongoing:

- The third Subject was safely treated with the low-dose of BB-301 in October 2024, and the fourth Subject is anticipated to receive the low-dose of BB-301 in December 2024.

Adverse Events:

- No Serious Adverse Events have been observed for the three Subjects treated with the low-dose of BB-301.

Corporate Updates:

- On October 12th the Principal Investigator of the BB-301 Phase 1b/2a Clinical Treatment Study shared updated interim results for two Subjects treated in the Phase 1b/2a Open-label, Dose Escalation Study of BB-301 in a late-breaking oral presentation at the 29th Annual Congress of the World Muscle Society, in Prague, Czech Republic.
- The Company held a webcast discussing the interim clinical study results on October 14th (the replay of this event is available here).

Financial Highlights

First Quarter 2025 Financial Results

Total Revenues for the quarter ended September 30, 2024, were $0 million compared to $0 revenues collected for the quarter ended September 30, 2023.

Total Expenses for the quarter ended September 30, 2024, were $5.8 million compared to $5.9 million for the quarter ended September 30, 2023. For the quarter ended September 30, 2024, the Company received no royalties and license fees compared to a royalties and license fee credit of $106,000 for the quarter ended September 30, 2023. The Company incurred $3.6 million of research and development expenses for the quarter ended September 30, 2024 compared to $4.4 million for the quarter ended September 30, 2023. Research and development expenses relate primarily to ongoing clinical development of BB-301 for the treatment of OPMD-related Dysphagia. General and administrative expenses were $2.2 million for the quarter ended September 30, 2024 compared to $1.6 million for the quarter ended September 30, 2023.

The loss from operations for the quarter ended September 30, 2024, was $5.2 million compared to a loss of $5.9 million for the quarter ended September 30, 2023. Net loss attributable to shareholders for the quarter ended September 30, 2024, was $5.1 million, or $(0.48) per basic and diluted share, compared to a net loss of $6.6 million, or $(3.05) per basic and diluted share for the quarter ended September 30, 2023. As of September 30, 2024, the Company had $67.8 million in cash and cash equivalents.

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

	September 30, 2024	June 30, 2024
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 67,841	$ 50,866
Restricted Cash	64	63
Trade and other receivables	4	229
Prepaid and other assets	426	516
Total current assets	68,335	51,674
Property and equipment, net	154	179
Deposits	25	25
Other assets	56	62
Right-of-use assets	204	270
Total assets	$ 68,774	$ 52,210
Liabilities and Stockholders' Equity		
Current liabilities:		
Trade and other payables	$ 3,847	$ 4,165
Accrued employee benefits	495	475
Lease liabilities, current portion	211	284
Total current liabilities	4,553	4,924
Non-current accrued employee benefits	41	38
Lease liabilities, less current portion	-	-
Total liabilities	4,594	4,962
Commitments and contingencies (Note 10)		
Stockholders' equity:		
Common stock, $0.0001 par value - 160,000,000 shares authorized; 17,893,765 and 10,086,119 shares issued and outstanding at September 30, 2024 and June 30, 2024, respectively	1	1
Additional paid-in capital	260,490	238,398
Accumulated deficit	(195,318)	(190,259)
Accumulated other comprehensive loss	(993)	(892)
Total stockholders' equity	64,180	47,248
Total liabilities and stockholders' equity	$ 68,774	$ 52,210

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

	Three Months Ended September 30,	
	2024	**2023**
Revenue:		
Licensing revenues from customers	$ -	$ -
Total revenues	-	-
Operating expenses		
Royalties and license fees	-	(106)
Research and development	3,585	4,429
General and administrative	2,206	1,551
Total operating expenses	5,791	5,874
Loss from operations	(5,791)	(5,874)
Other income (loss):		
Foreign currency transaction gain (loss)	93	(56)
Interest income (expense), net	604	(6)
Other income (expense), net	35	(18)
Unrealized gain (loss) on investment	-	-
Total other income (loss), net	732	(80)
Net loss	$ (5,059)	$ (5,954)
Other comprehensive income:		
Unrealized foreign currency translation gain (loss)	(101)	50
Total other comprehensive income	(101)	50
Total comprehensive loss	$ (5,160)	$ (5,904)
Net loss	$ (5,059)	$ (5,954)
Deemed dividends	-	(619)
Net loss attributable to common shareholders	$ (5,059)	$ (6,573)
Net loss per share:		
Basic and diluted	$ (0.48)	$ (3.05)
Weighted average number of shares outstanding: basic and diluted	10,644,533	2,157,065

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.

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, 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 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, 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 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.

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