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Benitec Biopharma Releases First Quarter 2023 Financial Results and Provides Operational Update

HAYWARD, Calif., Nov. 10, 2022 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) ("Benitec" or "the Company"), a development-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on its proprietary DNA-directed RNA interference ("ddRNAi") platform, today announced financial results for its Fiscal Year First Quarter ended September 30, 2022. The Company has filed its quarterly report on Form 10-Q for the quarter ended September 30, 2022, with the U.S. Securities and Exchange Commission.

"Following the receipt of a favorable decision from the Institutional Review Board of the lead clinical site for the OPMD Natural History Study in the United States and the formal completion of the Site Initiation Visit at this key clinical enrollment site, the screening of OPMD subjects for enrollment into the OPMD Natural History Study will begin this month. The initiation of subject screening for the OPMD Natural History Study represents a major milestone for Benitec, and we look forward to enrolling the first subjects into the OPMD Natural History Study this year. The enrollment of subjects into the OPMD Natural History Study in 2022 continues to support our central clinical development goal of administering the first dose of BB-301 in 2023," said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec Biopharma. "The Principal Investigator of the OPMD Natural History Study in the United States expects high enrollment interest for this incurable genetic disease with no approved treatments, and we continue to work with regulators globally to open additional sites in geographies outside of the United States."

Operational Updates

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

BB-301 Clinical Development Program Overview:

- The BB-301 clinical development program will be conducted in the United States, Canada, and France, and the primary elements of the clinical development plan are summarized below.
    - The clinical development plan will begin in 2022 and comprise approximately 76 weeks of follow-up:
        - 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.

- 1-day of BB-301 dosing to initiate participation in the Phase 1b/2a single-arm, open-label, sequential, dose-escalation cohort study.

- 52-weeks of post-dosing follow-up for conclusive evaluation of the primary and secondary endpoints of the Phase 1b/2a BB-301 treatment study.

- The OPMD Natural History (NH) Study (i.e., the 6-month pre-treatment observation period) will facilitate the characterization of OPMD patient disposition at baseline and assess subsequent rates of progression of dysphagia via the use of the following quantitative radiographic measures (i.e., videofluoroscopic swallowing studies or "VFSS"), with the VFSS outlined below collectively providing 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 $\%(C2\text{-}4)^2$
  - Pharyngeal Area at Maximum Constriction (PhAMPC)
  - Dynamic Imaging Grade of Swallowing Toxicity Scale (DIGEST)
  - Vallecular Residue $\%(C2\text{-}4)^2$, Pyriform Sinus Residue $\%(C2\text{-}4)^2$, and Other Pharyngeal Residue $\%(C2\text{-}4)^2$
  - 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 oropharyngeal dysphagia, along with two distinct patient-reported outcome instruments targeting the assessment of oropharyngeal dysphagia.

- Upon the achievement of 6-months of follow-up in the NH Study, participants will be eligible for enrollment onto the BB-301 Phase 1b/2a treatment study.

- BB-301 Phase 1b/2a Treatment Study:

  - This first-in-human (FIH) study will be a Phase 1b/2a, open-label, dose-escalation study to evaluate the safety and clinical activity of intramuscular doses of BB-301 administered to subjects with OPMD.

  - Upon rollover onto the Phase 1b/2a BB-301 treatment study, the follow-up of OPMD study participants will continue for 52 weeks.

  - The primary endpoints of the FIH study will be safety and tolerability, with secondary endpoints comprising quantitative radiographic measures of global swallowing function and pharyngeal constrictor muscle function (i.e., VFSS) as well as clinical assessments (all of which will be equivalent to those employed for the NH study). These endpoints will be evaluated during each 90-day period following BB-301 intramuscular injection (Day 1).

- The natural history of dysphagia observed for each OPMD study participant, as characterized by the VFSS and clinical swallowing assessments carried out during the NH Study, will serve as the baseline for comparative assessment of safety and efficacy of BB-301 upon rollover from the NH Study onto the Treatment Study.

Operational and Regulatory Updates for the Clinical Development Program:

North America:

- Formal submission of the comprehensive NH Study trial package to the Institutional Review Board (IRB) at the lead clinical site in the United States was completed in August 2022.
    - The submission package included: the NH Study Protocol, the subject Informed Consent Form, the patient-facing clinical documents and recruitment documents, the physician-facing clinical and laboratory manuals and recruitment documents.
    - Additionally, the primary clinical study site was formally qualified, the Principal Investigator and Sub-Investigators were identified at the clinical study site, the core VFSS site was selected and qualified, and the Central Reader for the VFSS data was selected.
- The IRB for the lead clinical site in the United States approved the NH Study trial package in September 2022.
    - Following the favorable IRB decision, the Site Initiation Visit was completed in October 2022, and the screening of OPMD subjects for enrollment into the OPMD Natural History Study will begin at the lead clinical site in the United States in November 2022.
- Planning for the formal submission of the comprehensive NH Study trial package to the Research Ethics Board (REB) for the lead clinical site in Canada is ongoing.
    - Approval of the overall NH Study trial package by the REB allows for clinical study site activation and OPMD patient screening and enrollment to begin in Canada.

France:

- In the Third Calendar Quarter 2022, Benitec submitted responses to the Central Ethics Committee (CEC) related to minor non-clinical queries.
- In November 2022 two additional non-clinical queries have been received from the CEC regarding the General Data Protection Regulation (GDPR) in the European Union.
    - GDPR is a regulation that requires businesses to protect the personal data and privacy of European Union (EU) citizens.

    - GDPR became effective on May 25, 2018, and is applicable to organizations within the EU that use personal data, as well as international organizations that provide goods and services to individuals in the EU or monitor their behavior.

- Approval of the overall NH Study trial package by the CEC allows for clinical study site activation and OPMD patient screening and enrollment to begin in France

BB-301 Phase 1b/2a Regulatory Updates:

- Investigational New Drug (IND) and Clinical Trial Application (CTA) filings are anticipated in the First Calendar Quarter 2023.

- IND and CTA filings are required to initiate the BB-301 Phase 1b/2a Treatment Study in the United States, Canada, and France.

- The first NH Study subject is anticipated to be eligible for BB-301 administration in the Second Calendar Quarter 2023 (following 6-months of NH Study enrollment and follow-up and final confirmation of eligibility for the BB-301 Phase 1b/2a Treatment Study).

- Interim safety and efficacy data for subjects enrolled onto the BB-301 Phase 1b/2a Treatment Study are anticipated to become available for disclosure approximately every 90-days following BB-301 administration.

    - In preclinical proof-of-concept studies for BB-301, the OPMD disease phenotype was reversed at 14 weeks (i.e., approximately 90-days) after the administration of BB-301 (with the strength of BB-301 injected muscles being restored in the diseased animals and robust evidence of anatomical and microscopic improvement of the BB-301 injected muscle tissues being observed).

Financial Highlights

First Quarter 2023 Financial Results

Total Revenues for the quarter ended September 30, 2022, were $0 compared to $0 for the quarter ended September 30, 2021.

Total Expenses for the quarter ended September 30, 2022, were $4.6 million compared to $4.8 million for the quarter ended September 30, 2021. The Company incurred $2.7 million of research and development expenses compared to $2.8 million for the comparable quarter ended September 30, 2021. Research and development expenses relate primarily to the OPMD project. The Company continued with the GMP
manufacturing project after concluding the BB-301 Regulatory Toxicology Study and the Parallel Assay Method Development, Qualification, and Validation project. For the quarter ended September 30, 2022, general and administrative expenses were $1.9 million compared to $2.0 million for the quarter ended September 30, 2021. The decrease was due to the decrease in salaries and wages, insurance, state and local taxes.

The loss from operations for the quarter ended September 30, 2022, was $4.6 million

compared to a loss of $4.8 million for the quarter ended September 30, 2021. Net loss attributable to shareholders for the quarter ended September 30, 2022, was $5.1 million, or $0.47 per basic and diluted share, compared to a net loss of $5.0 million, or $0.62 per basic and diluted share for the quarter ended September 30, 2021. As of September 30, 2022, the Company had $16.5 million in cash and cash equivalents.

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

	September 30, 2022	June 30, 2022
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 16,534	$ 4,062
Restricted cash	13	14
Trade and other receivables	3	3
Prepaid and other assets	531	741
Total current assets	17,081	4,820
Property and equipment, net	179	222
Deposits	25	25
Other assets	119	135
Right-of-use assets	711	771
Total assets	$ 18,115	$ 5,973
Liabilities and stockholders' equity		
Current liabilities:		
Trade and other payables	$ 2,368	$ 1,880
Accrued employee benefits	385	400
Lease liabilities, current portion	258	252
Total current liabilities	3,011	2,532
Lease liabilities, less current portion	491	559
Total liabilities	3,502	3,091
Commitments and contingencies (Note 10)		
Stockholders' equity:		
Common stock, $0.0001 par value-40,000,000 shares authorized; 25,809,533 shares and 8,171,690 shares issued and outstanding at September 30, 2022 and June 30, 2022, respectively	3	1
Additional paid-in capital	168,768	152,453
Accumulated deficit	(153,420)	(148,327)
Accumulated other comprehensive loss	(738)	(1,245)
Total stockholders' equity	14,613	2,882
Total liabilities and stockholders' equity	$ 18,115	$ 5,973

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

	Three Months Ended September 30	
	2022	2021
Revenue:		
Licensing revenues from customers		
Total revenues	$ -	$ -
	-	-
Operating Expenses		
Research and development	2,660	2,780
General and administrative	1,920	2,042
Total operating expenses	4,580	4,822
Loss from operations	(4,580)	(4,822)
Other income (loss):		
Foreign currency transaction loss	(507)	(240)
Interest expense, net	(9)	(1)
Unrealized gain on investment	3	18
Total other income (loss), net	(513)	(223)
Net loss	$ (5,093)	$ (5,045)
Other comprehensive income:		
Unrealized foreign currency translation gain	507	239
Total other comprehensive income	507	239
Total comprehensive loss	$ (4,586)	$ (4,806)
Net loss	$ (5,093)	$ (5,045)
Net loss per share: basic and diluted	$ (0.47)	$ (0.62)
Weighted average number of shares outstanding: basic and diluted	10,855,710	8,171,690

About Benitec Biopharma Inc.

Benitec Biopharma Inc. (“Benitec” or the “Company”) is a development-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 Company is developing ddRNAi-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 initiation and completion of pre-clinical and clinical trials, the timing of patient enrolment and dosing in clinical trials, the timing of expected regulatory filings, the clinical utility and potential attributes and benefits of ddRNAi and Benitec's product candidates, potential future out-licenses and collaborations, the intellectual property position and the ability to procure additional sources of financing, 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: 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; 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 Company's ability to satisfy its capital needs through increasing its revenue and obtaining additional financing; given market conditions and other factors, including our capital structure; our ability to continue as a going concern; the length of time over which the Company expects its cash and cash equivalents to be sufficient to execute on its business plan; the impact of the current COVID-19 pandemic, the disease caused by the SARS-CoV-2 virus, which may adversely impact the Company's business and pre-clinical and future clinical trials; 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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BENITEC BIOPHARMA
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Source: Benitec Biopharma Inc.