Prothena Reports First Quarter 2021 Financial Results and Provides Updated Financial Guidance and R&D Update

May 11, 2021

- **Net cash used in operating and investing activities was $33.7 million in the first quarter; quarter-end cash and restricted cash position of $345.7 million provides funding to advance the R&D pipeline**
- **Announced confirmatory Phase 3 AFFIRM-AL study of birtamimab in Mayo Stage IV patients with AL amyloidosis under SPA agreement with FDA**
- **Achieved $60 million milestone from Roche for first patient dosed in Phase 2b PADOVA study of prasinezumab in patients with early Parkinson's disease**
- **Highlighted data in oral presentations at AD/PD in March and AAN in April for three programs in our neurodegenerative and rare peripheral amyloid pipeline**

DUBLIN, Ireland, May 11, 2021 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a late-stage clinical company with a robust pipeline of novel investigational therapeutics built on protein dysregulation expertise, today reported financial results for the first quarter of 2021. In addition, the Company provided an update on its R&D programs and 2021 financial guidance.

"We recently welcomed Hideki Garren to Prothena as Chief Medical Officer and look forward to his expertise contributing to our transition to a fully integrated research, development and commercial biotechnology company," said Gene Kinney, Ph.D., President and Chief Executive Officer of Prothena. "The oral presentations at AD/PD and AAN highlight the continued translation of our differentiated protein dysregulation scientific platform from preclinical findings to clinical benefit for patients across multiple programs in our portfolio. We remain on track for significant news flow this year with the planned initiation of three late-stage clinical studies for birtamimab, prasinezumab and PRX004. We will advance two programs in our Alzheimer's portfolio towards the clinic over the next 12 months, with IND filings for our anti-tau PRX005 and anti-abeta PRX012 programs, designed to be best-in-class treatments for Alzheimer's disease. Additionally, our cash position was strengthened by our public offering in March and will be further bolstered when we receive the $60 million milestone earned from Roche. Together this enhances our ability to fund the company through multiple key clinical milestones."

First Quarter and Recent Highlights:

Birtamimab, a potential treatment for AL amyloidosis, is a humanized monoclonal antibody designed to directly neutralize soluble toxic aggregates and promote clearance of amyloid that causes organ dysfunction and failure

- Based on the significant survival benefit favoring birtamimab, from the previous VITAL study in a subset of patients categorized as Mayo Stage IV at baseline (HR=0.413, p=0.025, over 9 months), and multiple in-depth discussions with the U.S. Food and Drug Administration (FDA), Prothena announced plans in February 2021 to advance birtamimab into the confirmatory Phase 3 AFFIRM-AL study in Mayo Stage IV patients with AL amyloidosis. AFFIRM-AL is a global, registration-enabling Phase 3 study that will be conducted under a Special Protocol Assessment (SPA) agreement with FDA to enable registration at p≤0.10 for the primary endpoint of all-cause mortality.

Prasinezumab, a potential treatment for Parkinson's disease, is a humanized monoclonal antibody designed to target a key epitope within the C-terminus of alpha-synuclein and is the focus of the worldwide collaboration with Roche

- $60 million milestone earned with first patient dosed in PADOVA study in the second quarter
- Two oral presentations by Roche at the 15th International Conference on Alzheimer's and Parkinson's Diseases (AD/PD 2021) released new data from Part 1 of the Phase 2 PASADENA study. The first presentation highlighted newly presented data from a pre-specified exploratory subgroup analysis showing slowing of clinical decline with prasinezumab was more evident in subgroups with more rapid disease progression. Separately, new digital biomarker data presented from Roche's remote monitoring technology used in the study was consistent with a potential disease modifying effect of prasinezumab in slowing Parkinson's disease progression.

PRX004, a potential treatment for ATTR amyloidosis, is a humanized monoclonal antibody designed to deplete the pathogenic, non-native forms of the TTR protein

- Oral presentation at the American Academy of Neurology (AAN) 2021 Virtual Meeting featuring results from the Phase 1 study of PRX004 in ATTR amyloidosis. PRX004 showed favorable results as demonstrated by slowing of neuropathy progression for all 7 evaluable patients at 9 months, including improvement in neuropathy in 3 of the 7 patients, and improved cardiac systolic function for all 7 patients. In this Phase 1 study, PRX004 was found to be generally safe and well tolerated across all dose levels.

PRX005, a potential treatment for Alzheimer's disease (AD), is an investigational antibody that targets tau, a protein implicated in diseases including AD, frontotemporal dementia (FTD), progressive supranuclear palsy (PSP), chronic traumatic encephalopathy (CTE) and other tauopathies.

- Oral presentation at AD/PD 2021 highlighting new preclinical data demonstrating that targeting an epitope within the microtubule binding region (MTBR) of tau with PRX005 resulted in superior attributes for the potential treatment of AD. PRX005 demonstrated significant inhibition of cell-to-cell transmission and neuronal internalization in vitro and in vivo, and slowed pathological progression in a tau transgenic mouse model.

Corporate

- Appointed Hideki Garren, M.D., Ph.D., as Chief Medical Officer, responsible for leading the clinical and medical organizations to advance Prothena's clinical pipeline. Dr. Garren joined Prothena after serving as Vice President, Global Head of Neuroimmunology at F. Hoffman-La Roche Ltd. (Roche) & Genentech Inc.
- Executed a public offering in March that raised net proceeds of approximately $78 million through the issuance of 4,025,000 ordinary shares

Upcoming Milestones:

Birtamimab

- Phase 3 AFFIRM-AL study initiation expected mid-2021
- VITAL study 9-month results expected to be presented at a medical conference in 2021

Prasinezumab

- Results from Part 2 of the PASADENA study expected to be presented at an upcoming medical conference

PRX004

- Phase 2/3 study in patients with ATTR-cardiomyopathy expected to initiate 4Q 2021

PRX005

- IND expected by 3Q 2021
- $80 million potential payment from Bristol Myers Squibb upon exercising their US license option in 2021

PRX012, a potential treatment for Alzheimer's disease, is a high affinity monoclonal antibody targeting a key epitope within the N-terminus of Aβ

- IND expected by 1Q 2022

Upcoming Investor Conferences

Members of the senior management team will present and participate in investor meetings at the following upcoming investor conferences:

- BofA Securities 2021 Virtual Health Care Conference, May 13, 2021, at 8:45 AM ET
- RBC Capital Markets Global Healthcare Conference, May 19, 2021 at 8:35 AM ET
- Jefferies Virtual Healthcare Conference, June 1, 2021 at 9:00 AM ET

First Quarter 2021 Financial Results and Updated 2021 Financial Guidance

For the first quarter of 2021, Prothena reported a net loss of $36.7 million, as compared to a net loss of $23.6 million for the first quarter of 2020. Net loss per share for the first quarter of 2021 was $0.91, as compared to a net loss per share of $0.59 for the first quarter of 2020.

Prothena reported total revenue of $0.2 million for the first quarter of 2021, from collaboration and license revenue from Roche, as compared to total revenue of $0.1 million for the first quarter of 2020, from collaboration revenue from Roche.

Research and development (R&D) expenses totaled $21.1 million for the first quarter of 2021, as compared to $15.2 million for the first quarter of 2020. The increase in R&D expense for the first quarter of 2021 compared to the same period in the prior year was primarily due to higher R&D consulting expense, higher personnel expense, higher manufacturing expense primarily related to our PRX012 and birtamimab programs, higher clinical trial expense primarily related to birtamimab partially offset by lower PRX004 clinical trial expenses and higher collaboration expense with Roche related to the prasinezumab program. R&D expenses included non-cash share-based compensation expense of $2.0 million for the first quarter of 2021, as compared to $2.0 million for the first quarter of 2020.

General and administrative (G&A) expenses totaled $11.1 million for the first quarter of 2021, as compared to $9.7 million for the first quarter of 2020. The increase in G&A expenses for the first quarter of 2021 compared to the same period in the prior year was primarily related to higher personnel expense and higher expense for our directors and officers insurance premium. G&A expenses included non-cash share-based compensation expense of $4.2 million for the first quarter of 2021, as compared to $3.5 million for the first quarter of 2020.

Total non-cash share-based compensation expense was $6.2 million for the first quarter of 2021, as compared to $5.5 million for the first quarter of 2020.

As of March 31, 2021, Prothena had $345.7 million in cash, cash equivalents and restricted cash (does not include the $60 million milestone earned in the second quarter from Roche) and no debt.

As of May 4, 2021, Prothena had approximately 44.2 million ordinary shares outstanding.

The Company continues to expect the full year 2021 net cash used in operating and investing activities to be $51 to $74 million, which includes receiving the $60 million milestone earned in the second quarter from Roche. The Company is updating its projected year end cash balance to approximately $316 million in cash, cash equivalents and restricted cash (midpoint) (versus prior guidance of $235 million) to include an additional $81 million in net proceeds primarily from the public offering in March. The estimated full year 2021 net cash used in operating and investing activities is primarily driven by an estimated net loss of $79 to $111 million, which includes an estimated $20 million of non-cash share-based compensation expense.

About Prothena

Prothena Corporation plc is a late-stage clinical company with a robust pipeline of novel investigational therapeutics built on protein dysregulation expertise with the potential to change the course of devastating rare peripheral amyloid and neurodegenerative diseases. Fueled by its deep scientific expertise built over decades of research, Prothena is advancing a pipeline of therapeutic candidates for a number of indications and novel targets for which its ability to integrate scientific insights around neurological dysfunction and the biology of misfolded proteins can be leveraged. Prothena's pipeline includes both wholly-owned and partnered programs being developed for the potential treatment of diseases including AL amyloidosis, ATTR amyloidosis, Alzheimer's disease, Parkinson's disease and a number of other neurodegenerative diseases. For more information, please visit the Company's website at www.prothena.com and follow the Company on Twitter @ProthenaCorp.

Forward-looking Statements

This press release contains forward-looking statements. These statements relate to, among other things, the sufficiency of our cash position to fund advancement of a broad pipeline; our goal of building a protein dysregulation platform; the treatment potential, designs, and proposed mechanisms of action of birtamimab, prasinezumab, PRX004, PRX005, and PRX012; plans for future clinical studies of birtamimab, prasinezumab, PRX004, PRX005, and PRX012; amounts we might receive under our collaborations with Roche and Bristol Myers Squibb; the expected timing of reporting data from prior clinical studies of birtamimab and the Phase 2 clinical study of prasinezumab; our anticipated net cash burn from operating and investing activities for 2021 and expected cash balance at the end of 2021; and our estimated net loss and non-cash share-based compensation expense for 2021. These statements are based on estimates, projections and assumptions that may prove not to be accurate, and actual results could differ materially from those anticipated due to known and unknown risks, uncertainties and other factors, including but not limited to the effects on our business of the worldwide COVID-19 pandemic and the risks, uncertainties and other factors described in the "Risk Factors" sections of our Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 11, 2021, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the SEC. We undertake no obligation to update publicly any forward-looking statements contained in this press release as a result of new information, future events, or changes in our expectations.

PROTHENA CORPORATION PLC
CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited - amounts in thousands except per share data)

	Three Months Ended March 31,	
	2021	**2020**
Collaboration revenue	$ 110	$ 141
License revenue	50	—
Total revenue	160	141
Operating expenses:		
Research and development	21,144	15,248
General and administrative	11,125	9,741
Total operating expenses	32,269	24,989
Loss from operations	(32,109)	(24,848)
Other income, net	34	1,113
Loss before income taxes	(32,075)	(23,735)
Provision for (benefit from) income taxes	4,660	(166)
Net loss	$ (36,735)	$ (23,569)
Basic and diluted net loss per share	$ (0.91)	$ (0.59)
Shares used to compute basic and diluted net loss per share	40,250	39,909

PROTHENA CORPORATION PLC
CONSOLIDATED BALANCE SHEETS
(unaudited - amounts in thousands)

	March 31, 2021	**December 31, 2020**
Assets		
Cash and cash equivalents	$ 342,993	$ 295,380

Accounts receivable	1	15
Prepaid expenses and other current assets	7,955	2,537
Restricted cash, current	1,352	1,352
Total current assets	352,301	299,284
Property and equipment, net	2,295	2,551
Operating lease right-of-use assets	16,411	17,811
Restricted cash, non-current	1,352	1,352
Other non-current assets	7,360	11,977
Total non-current assets	27,418	33,691
Total assets	$ 379,719	$ 332,975
Liabilities and Shareholders' Equity		
Accrued research and development	9,355	9,044
Lease liability, current	5,617	5,512
Other current liabilities	8,484	11,292
Total current liabilities	23,456	25,848
Deferred revenue, non current	110,242	110,242
Lease liability, non-current	10,884	12,326
Other non-current liabilities	553	553
Total non-current liabilities	121,679	123,121
Total liabilities	145,135	148,969
Total shareholders' equity	234,584	184,006
Total liabilities and shareholders' equity	$ 379,719	$ 332,975

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Source: Prothena Corporation plc