



Prothena Reports Fourth Quarter and Full Year 2019 Financial Results, and Provides Financial Guidance and R&D Update

February 12, 2020

- **Net cash used in operating and investing activities was \$14.6 million in the fourth quarter and \$53.5 million for the full year 2019; quarter-end cash and restricted cash position of \$378.4 million provides funding to advance a broad pipeline**
- **Reported interim data from Phase 1 study of PRX004 in patients with hereditary ATTR amyloidosis**

DUBLIN, Ireland, Feb. 12, 2020 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a clinical-stage neuroscience company with expertise in protein misfolding, today reported financial results for the fourth quarter and full year 2019. In addition, the Company provided 2020 financial guidance and an update on its R&D programs.

"In 2019, we continued to advance our pipeline of novel investigational therapeutics towards key milestones," said Gene Kinney, Ph.D., President and Chief Executive Officer of Prothena. "We reported interim data from our first-in-human Phase 1 study of PRX004 in patients with hereditary ATTR amyloidosis and continue to expect data from Part 1 of the Phase 2 PASADENA study of prasinezumab in patients with early Parkinson's disease this year. Looking ahead, we have expanded our innovative discovery and preclinical pipeline which consists of three targets under our global neuroscience collaboration with Bristol-Myers Squibb, as well as our proprietary preclinical and discovery programs that comprise our Alzheimer's disease portfolio."

Full Year 2019 and Recent Developments:

Prasinezumab (PRX002/RG7935), a potential treatment for Parkinson's disease, is a monoclonal antibody designed to target alpha-synuclein and is the focus of the worldwide collaboration with Roche

- Completed last patient visit in part 1 of the two-part Phase 2 PASADENA study being conducted by Roche in patients with early Parkinson's disease (N=316).

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

- Reported interim data from the first-in-human dosing in a Phase 1 clinical study of PRX004 in patients with hereditary ATTR (hATTR) amyloidosis. In the interim analysis, 15 patients in the dose escalation phase of the study each received 3 infusions in dose-level cohorts 1 through 5 representing 0.1, 0.3, 1.0, 3.0 and 10.0 mg/kg. PRX004 was found to be generally safe and well tolerated and demonstrated pharmacokinetic profiles consistent with that of an immunoglobulin gamma 1 (IgG1) monoclonal antibody. Target engagement was demonstrated by a dose-dependent decrease in plasma levels of misTTR (the non-native forms of TTR), as measured by our proprietary misTTR biomarker assay. For the three patients in the 10.0 mg/kg dose-level (the highest dose-level in the interim analysis), the maximum observed reductions in misTTR levels occurred within 24 hours of the first infusion were 54%, 66% and 76%. As expected, because PRX004 was designed to recognize an epitope exposed only on the misTTR species, PRX004 did not appear to impact levels of normal tetrameric TTR.

Discovery and Preclinical Development: Prothena is advancing an early-stage pipeline of programs for a number of potential neurological indications

- Initiated cell line development of a lead candidate in the preclinical tau program, part of a worldwide collaboration with Bristol-Myers Squibb, in the second quarter of 2019.
- Initiated cell line development of a lead candidate in the proprietary preclinical A β (Amyloid beta) program in the fourth quarter of 2019.

Corporate

- Appointed Paula Cobb to the Board of Directors. Ms. Cobb is a biotechnology executive with wide-ranging global development, business strategy and commercial experience across a number of categories. She is the former Chief Operating Officer of Decibel Therapeutics and held several senior global leadership positions at Biogen, including as Senior Vice President of the rare disease group.
- Appointed Oleg Nodelman to the Board of Directors. With more than 20 years of biotech investing experience, Mr.

Nodelman is a veteran biotechnology investor and advisor with deep roots in the biotechnology and scientific communities. He is the founder and portfolio manager of EcoR1 Capital LLC, a biotech-focused investment advisory firm established in 2013. Before founding EcoR1, Mr. Nodelman was a portfolio manager at BVF Partners, one of the first hedge funds dedicated to the biotechnology sector.

Upcoming Research and Development Milestones

Prasinezumab

- The Phase 2 PASADENA study in patients with early Parkinson's disease (N=316) is being conducted by Roche, and data from Part 1 of this study are expected to be reported in 2020

PRX004

- Interim data from the ongoing Phase 1 study was reported in December 2019, and additional data from the dose-escalation and long-term extension portions of the study are expected to be reported in 2020

Preclinical

- The preclinical tau program, part of a global neuroscience collaboration with Bristol-Myers Squibb, is expected to advance IND-enabling activities in 2020
- The preclinical A β program is expected to initiate IND-enabling studies in 2020

Fourth Quarter and Full Year of 2019 Financial Results and 2020 Financial Guidance

For the fourth quarter and full year of 2019, Prothena reported a net loss of \$21.6 million and \$77.7 million, respectively, as compared to a net loss of \$22.5 million and \$155.6 million for the fourth quarter and full year of 2018, respectively, which included a restructuring credit of \$1.6 million in the fourth quarter of 2018 and restructuring charges of \$16.1 million for the full year of 2018 associated with the discontinuation of the NEOD001 program. Net loss per share for the fourth quarter and full year of 2019 was \$0.54 and \$1.95, respectively, as compared to a net loss per share of \$0.56 and \$3.93 for the fourth quarter and full year of 2018, respectively.

Prothena reported total revenue, all from its collaboration with Roche, of \$0.3 million and \$0.8 million for the fourth quarter and full year of 2019, respectively, as compared to total revenue of \$0.2 million and \$1.0 million for the fourth quarter and full year of 2018, respectively.

Research and development (R&D) expenses totaled \$15.5 million and \$50.8 million for the fourth quarter and full year of 2019, respectively, as compared to \$16.5 million and \$101.2 million for the fourth quarter and full year of 2018, respectively. The decrease in R&D expense for the fourth quarter of 2019 compared to the same period in the prior year was primarily due to lower intellectual property license expense, lower clinical costs (primarily associated with the discontinuation of the NEOD001 program partially offset by higher costs for the PRX004 program) and lower personnel costs (including share-based compensation expense); offset in part by higher collaboration expense with Roche. The decrease in R&D expense for the full year ended 2019 compared to the same period in the prior year was primarily due to lower clinical costs (primarily associated with the discontinuation of the NEOD001 program partially offset by higher costs for the PRX004 program), lower personnel costs (including share-based compensation expense), lower consulting costs and lower manufacturing costs (primarily associated with the discontinuation of the NEOD001 program and to a lesser extent to declines from the PRX004 program, offset in part by increase in cost for the tau program). R&D expenses included non-cash share-based compensation expense of \$2.0 million and \$8.1 million for the fourth quarter and full year of 2019, respectively, as compared to \$2.1 million and \$9.8 million for the fourth quarter and full year of 2018, respectively.

General and administrative (G&A) expenses totaled \$8.1 million and \$35.7 million for the fourth quarter and full year of 2019, respectively, as compared to \$8.0 million and \$42.5 million for fourth quarter and full year of 2018, respectively. G&A expenses for the fourth quarter of 2019 increased slightly compared to the same period in the prior year primarily related to higher lease cost recorded as operating expenses due to the adoption of the new lease standard (ASC 842) and higher directors and officer's insurance largely offset by other G&A cost. The decrease in G&A expenses for the full year of 2019 compared to the same period in the prior year was primarily due to lower personnel costs (including share-based compensation expense), receipt of sublease rental income, lower legal and accounting fees, lower depreciation and other expenses; offset in part by higher lease costs recorded as operating expenses due to the adoption of ASC 842. G&A expenses included non-cash share-based compensation expense of \$3.3 million and \$15.5 million for the fourth quarter and full year of 2019, respectively, as compared to \$3.7 million and \$16.2 million for the fourth quarter and full year of 2018, respectively.

Restructuring credit was nil and \$0.1 million for the fourth quarter and full year of 2019, respectively, as compared to \$1.6 million for the fourth quarter of 2018 and restructuring charges of \$16.1 million for the full year of 2018. The restructuring credit in 2019 was primarily the result of an adjustment in previously recorded employee termination benefits.

Total non-cash share-based compensation expense was \$5.3 million and \$23.6 million for the fourth quarter and full year of 2019, respectively, as compared to \$4.2 million and \$27.0 million for the fourth quarter and full year of 2018, respectively, which included a \$1.6 million share-based compensation credit and \$0.9 million share-based compensation expense included in the restructuring costs for the fourth quarter of 2018 and the full year of 2018, respectively.

As of December 31, 2019, Prothena had \$378.4 million in cash, cash equivalents and restricted cash and no debt.

As of February 7, 2020, Prothena had approximately 39.9 million ordinary shares outstanding.

The Company expects its full year 2020 net cash burn from operating and investing activities to be \$60-\$76 million, and to end the year with approximately \$310 million in cash, cash equivalents and restricted cash (midpoint). The estimated full year 2020 cash burn from operating and

investing activities is primarily driven by an estimated net loss of \$84-\$106 million, which includes an estimated \$23 million of non-cash shared-based compensation expense.

Conference Call Details

Prothena management will discuss these results and its 2020 financial guidance during a live audio conference call today, Wednesday, February 12, 2020, at 4:30 PM ET. The conference call will be made available on the Company's website at www.prothena.com under the Investors tab in the Events and Presentations section. Following the live audio webcast, a replay will be available on the Company's website for at least 90 days.

To access the call via dial-in, please dial (877) 887-5215 (U.S. and Canada toll free) or (315) 625-3069 (international) five minutes prior to the start time and refer to conference ID number 1758808. A replay of the call will be available until February 26, 2020 via dial-in at (855) 859-2056 (U.S. toll free) or (404) 537-3406 (international), Conference ID Number 1758808.

About Prothena

Prothena Corporation plc is a clinical-stage neuroscience company with expertise in protein misfolding, focused on the discovery and development of novel therapies with the potential to fundamentally change the course of devastating 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 partnered programs include prasinezumab (PRX002/RG7935), in collaboration with Roche for the potential treatment of Parkinson's disease and other related synucleinopathies, and programs that target tau, TDP-43 and an undisclosed target in collaboration with Bristol-Myers Squibb for the potential treatment of Alzheimer's disease, amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD) or other neurodegenerative diseases. Our proprietary programs include PRX004 for the potential treatment of ATTR amyloidosis, and programs that target A β (Amyloid beta) for the potential treatment of Alzheimer's disease.

For more information, please visit the Company's website at www.prothena.com and follow us 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; the treatment potential and proposed mechanisms of action of prasinezumab and PRX004; the expected timing of reporting data from the Phase 1 clinical study of PRX004; the expected timing of reporting data from the Phase 2 clinical study of prasinezumab; the continued advancement of our discovery and preclinical pipeline; and the timing of IND-enabling activities from our tau and A β programs; our anticipated net cash burn from operating and investing activities for 2020 and expected cash balance at the end of 2020; and our estimated net loss and non-cash share-based compensation expense for 2020. 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 risks, uncertainties and other factors described in the "Risk Factors" sections of our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 15, 2019, our subsequent Quarterly Reports on Form 10-Q filed with the SEC and our Annual Report on Form 10-K to be filed with the SEC for our fiscal year 2019. Prothena undertakes 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 Prothena's expectations.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Collaboration revenue	\$ 256	\$ 194	\$ 814	\$ 955
Total revenue	256	194	814	955
Operating expenses:				
Research and development	15,471	16,510	50,836	101,183
General and administrative	8,059	8,026	35,736	42,482
Restructuring and related impairment charges (credits)	—	(1,587)	(61)	16,145
Total operating expenses	23,530	22,949	86,511	159,810
Loss from operations	(23,274)	(22,755)	(85,697)	(158,855)
Other income, net	1,589	845	8,399	2,740
Loss before income taxes	(21,685)	(21,910)	(77,298)	(156,115)
Provision for (benefit from) income taxes	(131)	551	379	(470)
Net loss	\$(21,554)	\$(22,461)	\$(77,677)	\$(155,645)
Basic and diluted net loss per share	\$(0.54)	\$(0.56)	\$(1.95)	\$(3.93)
Shares used to compute basic and diluted net loss per share	39,897	39,864	39,882	39,559

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

December 31,
2019 2018

Assets

Cash and cash equivalents	\$ 375,723	\$ 427,659
Prepaid expenses and other current assets	2,652	3,731
Total current assets	378,375	431,390
Property and equipment, net	3,874	52,835
Operating lease right-of-use assets	23,274	—
Restricted cash, non-current	2,704	4,056
Other non-current assets	11,041	10,515
Total non-current assets	40,893	67,406
Total assets	\$ 419,268	\$ 498,796

Liabilities and Shareholders' Equity

Accrued research and development	\$ 5,826	\$ 5,370
Restructuring liability	—	461
Lease liability, current	5,101	—
Other current liabilities	6,787	9,095
Total current liabilities	17,714	14,926
Deferred revenue	110,242	110,242
Lease liability, non-current	17,838	—
Other non-current liabilities	553	50,630
Total non-current liabilities	128,633	160,872
Total liabilities	146,347	175,798
Total shareholders' equity	272,921	322,998
Total liabilities and shareholders' equity	\$ 419,268	\$ 498,796

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Source: Prothena Corporation plc