



Prothena Reports Fourth Quarter and Full Year 2018 Financial Results, and Provides Financial Guidance and R&D Update

February 14, 2019

- **Net cash used in operating and investing activities was \$22.5 million in the fourth quarter and \$30.0 million for the full year 2018; quarter-end cash and restricted cash position of \$431.7 million provides funding to advance neuroscience pipeline**
- **Entered into global neuroscience research & development collaboration with Celgene to advance novel therapies for patients with neurodegenerative diseases**
- **Initiated a first-in-human study of PRX004 in patients with ATTR amyloidosis**
- **Enrollment completed for the Phase 2 PASADENA study of prasinezumab in patients with early Parkinson's disease**

DUBLIN, Ireland, Feb. 14, 2019 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a clinical-stage neuroscience company, today reported financial results for the fourth quarter and full year 2018. In addition, the Company provided 2019 financial guidance and an update on its R&D programs.

"In 2018, we continued to advance our neuroscience pipeline of novel investigational therapeutics towards key milestones," said Gene Kinney, Ph.D., President and Chief Executive Officer of Prothena. "We initiated a first-in-human Phase 1 study of PRX004 in patients with hereditary ATTR amyloidosis and remain on track to report preliminary data later this year. In addition, enrollment in the Phase 2 PASADENA study of prasinezumab in patients with early Parkinson's disease has been completed by our colleagues at Roche, and we expect data from this study in 2020. Looking ahead, we continue to advance our discovery pipeline of novel targets under our collaboration with Celgene, as well as our proprietary and differentiated program in abeta, and expect to initiate cell line development of a lead candidate for both our tau and abeta programs this year."

Full Year 2018 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

- Published results from the Phase 1b multiple ascending dose study of prasinezumab in patients with Parkinson's disease in [JAMA Neurology](#). The paper is entitled "Safety and Tolerability of Multiple Ascending Doses of PRX002/RG7935, an Anti- α -Synuclein Monoclonal Antibody, in Patients With Parkinson Disease: A Randomized Clinical Trial."
- Enrollment completed (N=316) in the Phase 2 PASADENA study being conducted by Roche in patients with early Parkinson's disease.

PRX004, a potential treatment for ATTR amyloidosis, is a monoclonal antibody designed to target and clear the pathogenic, non-native forms of the TTR protein (misTTR) without affecting the native, or normal tetrameric form of the protein

- Achieved first-in-human dosing in a Phase 1 clinical study of PRX004 in patients with hereditary ATTR (hATTR) amyloidosis. The Phase 1 study will inform possible future studies and will include the use of Prothena's proprietary misTTR assay as a pharmacodynamic measure of the levels of non-native TTR species in plasma of patients across multiple hereditary TTR mutations.
- Presented a broad range of [research](#) in oral and poster presentations at the 16th International Symposium on Amyloidosis (ISA). New research was presented at ISA relating to PRX004 for the potential treatment of ATTR amyloidosis, including on our proprietary misTTR assay as well as preclinical research demonstrating that conformation-specific antibodies target misTTR and induce immune mediated clearance through phagocytosis.

Discovery: Prothena is advancing a research pipeline of novel targets for a number of potential neurological indications

- Entered into a [global neuroscience research & development collaboration](#) with Celgene to develop new therapies for a broad range of neurodegenerative diseases. The collaboration is focused on three targets implicated in the pathogenesis of several neurodegenerative diseases, including tau, TDP-43 and a third that is undisclosed. Prothena received a \$100 million upfront payment and a \$50 million equity investment by Celgene and is eligible to receive future potential exercise payments and regulatory and commercial milestones for each licensed program. Prothena is also eligible to receive additional royalties on net sales of any resulting marketed product.

- Entered into a multi-target license and option agreement with Bioasis Technologies Inc. Under the agreement, Prothena made an upfront payment of \$1 million to Bioasis and is exploring the application of Bioasis's xB³ platform technology to increase the delivery of therapeutics across the blood-brain barrier (BBB) for neuroscience disorders.

Corporate

- Appointed Radhika Tripuraneni, MD, MPH as Chief Development Officer. Dr. Tripuraneni is the Company's lead medical clinician, managing the clinical development, clinical operations, medical affairs and biometrics functions. She joined Prothena in 2018 as Vice President, Medical Affairs & Development Operations. Dr. Tripuraneni trained as a surgeon and has extensive biotech and investment industry experience across multiple therapeutic categories.
- Appointed [Tran B. Nguyen](#) as Chief Operating Officer, in addition to his responsibilities as Chief Financial Officer. Mr. Nguyen has been Prothena's Chief Financial Officer since 2013 and has more than 20 years of experience including strategic leadership positions in the biotech, banking and private equity industries.

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 in 2020

PRX004

- The Phase 1 study of PRX004 continues to enroll patients with hATTR amyloidosis, and preliminary data from lower dose cohorts, including safety, tolerability and pharmacodynamics, are expected in the fourth quarter of 2019

Discovery

- The preclinical tau program, part of a worldwide collaboration with Celgene, is expected to initiate cell line development of a lead candidate in 2019
- The preclinical A β (Amyloid beta) program is expected to initiate cell line development of a lead candidate in 2019 and to communicate preclinical data in the fourth quarter of 2019

Fourth Quarter and Full Year of 2018 Financial Results and 2019 Financial Guidance

Prothena reported a net loss of \$22.5 million and \$155.6 million, for the fourth quarter and full year of 2018, respectively, which includes a restructuring credit of \$1.6 million for 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 in April 2018, as compared to a net loss of \$47.8 million and \$153.2 million for the fourth quarter and the full year of 2017, respectively. Net loss per share for the fourth quarter and for the full year of 2018 was \$0.56 and \$3.93, respectively, as compared to a net loss per share of \$1.24 and \$4.07 for the fourth quarter and full year of 2017, respectively.

Prothena reported total revenue from its collaboration with Roche of \$0.2 million and \$1.0 million for the fourth quarter and full year of 2018, respectively, as compared to total revenue of \$0.2 million and \$27.5 million for the fourth quarter and full year of 2017, respectively. The decrease in revenue for the full year was primarily due to achievement of a clinical milestone from Roche of \$30.0 million in 2017 (of which \$26.6 million was recognized as collaboration revenue and \$3.4 million was recognized as an offset to R&D expenses) with no corresponding amount in 2018.

Research and development (R&D) expenses totaled \$16.5 million and \$101.2 million for the fourth quarter and full year of 2018, respectively, as compared to \$33.5 million and \$134.5 million for the fourth quarter and full year of 2017, respectively. The decrease in R&D expenses for the fourth quarter and full year of 2018 compared to fourth quarter and full year of 2017 was primarily due to lower product manufacturing expenses, lower clinical trial costs and lower personnel and consulting costs, offset in part by higher expenses associated with prasinezumab. R&D expenses included non-cash share-based compensation expense of \$2.1 million and \$9.8 million for the fourth quarter and full year of 2018, respectively, as compared to \$3.1 million and \$10.9 million for the fourth quarter and full year of 2017, respectively.

General and administrative (G&A) expenses totaled \$8.0 million and \$42.5 million for the fourth quarter and full year of 2018, respectively, as compared to \$14.0 million and \$48.2 million for the fourth quarter and full year of 2017, respectively. The decrease in G&A expenses for the fourth quarter and full year compared to the same periods in the prior year was primarily due to lower personnel costs and consulting expenses. G&A expenses included non-cash share-based compensation expense of \$3.7 million and \$16.2 million for the fourth quarter and full year of 2018, respectively, as compared to \$4.4 million and \$15.9 million for the fourth quarter and full year of 2017, respectively.

Restructuring credit totaled \$1.6 million for the fourth quarter of 2018 and restructuring charges totaled \$16.1 million for the full year of 2018. The restructuring credit in the fourth quarter was primarily the result of an adjustment in previously recorded employee termination benefits offset in part by asset impairment related to exiting our office lease in Ireland. Restructuring charges included non-cash share-based compensation credit of \$1.6 million for the fourth quarter of 2018 and non-cash share-based compensation expense of \$0.9 million for the full year of 2018, with no corresponding amounts in the fourth quarter or full year of 2017.

Total non-cash share-based compensation expense was \$4.2 million and \$27.0 million for the fourth quarter and full year of 2018, respectively, as compared to \$7.4 million and \$26.8 million for the fourth quarter and full year of 2017, respectively.

As of December 31, 2018, Prothena had \$431.7 million in cash, cash equivalents and restricted cash and no debt.

As of February 8, 2019, Prothena had approximately 39.9 million ordinary shares outstanding.

The Company expects the full year 2019 net cash burn from operating and investing activities to be \$64 million to \$72 million, and to end the year with approximately \$364 million in cash (mid-point). The estimated full year 2019 net cash burn from operating and investing activities is primarily driven by an estimated net loss of \$93 million to \$104 million, which includes an estimated \$26 million of non-cash share-based compensation expense.

Conference Call Details

Prothena management will discuss these results and its 2019 financial guidance during a live audio webcast and conference call today, Thursday, February 14, 2019, at 4:30 PM ET. The webcast 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 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 3791173. A replay of the call will be available until February 28, 2019 via dial-in at (855) 859-2056 (U.S. toll free) or (404) 537-3406 (international), Conference ID Number 3791173.

About Prothena

Prothena Corporation plc is a clinical-stage neuroscience company focused on the discovery and development of novel therapies with the potential to fundamentally change the course of progressive, life-threatening diseases. Fueled by its deep scientific understanding built over decades of neuroscience research, Prothena is advancing a pipeline of therapeutic candidates for a number of indications and novel targets including Parkinson's disease and other related synucleinopathies (prasinezumab - PRX002/RG7935) and ATTR amyloidosis (PRX004), as well as tau, A β (Amyloid beta) and TDP-43 where its scientific understanding of disease pathology can be leveraged. 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 our neuroscience 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 pipeline; the timing of initiating cell line development from our tau and abeta programs, and the timing of communicating preclinical data from the abeta program; the potential to receive future exercise payments, regulatory and commercial milestones and royalties under the Celgene collaboration; our anticipated net cash burn from operating and investing activities for 2019 and expected cash balance at the end of 2019; and our estimated net loss and non-cash share-based compensation expense for 2019. 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 February 27, 2017, 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 2018. 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,	
	2018	2017	2018	2017
Collaboration revenue	\$ 194	\$ 229	\$ 955	\$ 27,519
Total revenue	194	229	955	27,519
Operating expenses:				
Research and development	16,510	33,502	101,183	134,547
General and administrative	8,026	14,044	42,482	48,226
Restructuring and related impairment charges	(1,587)	—	16,145	—
Total operating expenses	22,949	47,546	159,810	182,773
Loss from operations	(22,755)	(47,317)	(158,855)	(155,254)
Other income (expense), net	845	(154)	2,740	(2,349)
Loss before income taxes	(21,910)	(47,471)	(156,115)	(157,603)
Provision for (benefit from) income taxes	551	287	(470)	(4,366)
Net loss	\$ (22,461)	\$ (47,758)	\$ (156,645)	\$ (164,319)
Basic and diluted net loss per share	\$ (0.56)	\$ (1.24)	\$ (3.93)	\$ (4.07)
Shares used to compute basic and diluted net loss per share	39,864	38,455	39,559	37,654

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

	December 31, 2018	December 31, 2017
Assets		
Cash and cash equivalents	\$ 427,659	\$ 417,620
Accounts receivable	2	240
Other current assets	3,729	8,467
Total current assets	431,390	426,327
Property and equipment, net	52,835	54,990
Restricted cash	4,056	4,056
Other assets	10,515	10,956
Total non-current assets	67,406	70,002
Total assets	\$ 498,796	\$ 496,329
Liabilities and Shareholders' Equity		
Accrued research and development	\$ 5,370	\$ 13,509
Restructuring liability	461	—
Other current liabilities	9,095	23,862
Total current liabilities	14,926	37,371
Deferred revenue	110,242	—
Other non-current liabilities	50,630	51,769
Total non-current liabilities	160,872	51,769
Total liabilities	175,798	89,140
Total shareholders' equity	322,998	407,189
Total liabilities and shareholders' equity	\$ 498,796	\$ 496,329

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Source: Prothena Corporation plc