



August 2, 2016

## Prothena Reports Second Quarter 2016 Financial Results; Provides R&D Update

- | **Net cash used in operating and investing activities was \$32.3 million in the second quarter and \$57.7 million for the first six months of 2016; strong quarter-end cash and restricted cash position of \$447.0 million supports advancement of multiple clinical development programs**
- | **Presented new clinical data from NEOD001 Phase 1/2 dose-escalation and expansion study, demonstrating improvement in three organ systems (cardiac, renal, and peripheral nerve) in previously-treated patients with AL amyloidosis**
- | **Presented clinical results from PRX003 Phase 1 single ascending dose study, demonstrating target engagement of novel anti-MCAM antibody for inflammatory diseases**

DUBLIN, Ireland, Aug. 02, 2016 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a late-stage clinical biotechnology company focused on the discovery, development and commercialization of novel protein immunotherapies, today reported financial results for the second quarter and first six months of 2016. In addition, the Company provided an update on its R&D programs and 2016 financial guidance.

"During the second quarter, we demonstrated positive momentum in our pipeline with oral presentations on new clinical data from two of our lead programs, NEOD001 and PRX003, at medical conferences," said Dale Schenk, PhD, President and Chief Executive Officer of Prothena. "The new clinical data from our NEOD001 Phase 1/2 dose-escalation and expansion study gives us continued confidence in the design and powering of the Phase 2b PRONTO and Phase 3 VITAL studies, which remain on track to our previously guided timelines. We continue to invest in our pipeline, and our financial position enables us to advance our programs through key milestones. During the coming 12 months we expect to announce topline results from the Phase 1b study of PRX002 in patients with Parkinson's disease, share interim results from the Phase 1b proof-of-biology study of PRX003 in patients with psoriasis, complete enrollment in the VITAL Amyloidosis Study of NEOD001, and advance PRX004 targeting transthyretin amyloid towards clinical development."

### Recent Highlights and Program Updates:

- | In an oral session at the 15th International Symposium on Amyloidosis (ISA), Morie A. Gertz, MD, of Mayo Clinic, presented [new interim clinical data](#) as of May 9, 2016 from 69 patients enrolled in the NEOD001 Phase 1/2 dose-escalation and expansion study, demonstrating best response rates of 53% and 63% for cardiac- (n=36) and renal- (n=35) evaluable patients, respectively. These cardiac and renal best response rates are consistent with results from the interim analysis of the dose-escalation phase of the Phase 1/2 study published in the Journal of Clinical Oncology in February 2016. In addition, improvement in peripheral neuropathy in patients in the prospectively defined peripheral neuropathy expansion cohort (n=11) was demonstrated by a mean 35% (median 23%) decrease in the Neuropathy Impairment Score-Lower Limb (NIS-LL) measured at month 10, leading to an 82% response rate and indicating improvement to a third organ system in NEOD001-treated patients. Two patients in this cohort demonstrated complete resolution of their peripheral neuropathy, as measured by NIS-LL. NEOD001 continued to be safe and well tolerated.
- | Also at ISA, presented preclinical research from Prothena's newest pipeline program, PRX004 for the potential treatment of TTR amyloidosis. An additional 7 posters and 1 oral session [at ISA included](#) research on new amyloid targets, and data on quality of life in AL amyloidosis.
- | In an oral session at the European League Against Rheumatism (EULAR) 17<sup>th</sup> Annual European Congress of Rheumatology, presented [positive results](#) from a Phase 1 clinical study of PRX003 in healthy volunteers that demonstrated PRX003 was safe and well-tolerated following a single infusion, up to and including the highest dose level tested of 30 mg/kg. Results from this study showed that administration of PRX003 led to greater than 95 percent neutralization of MCAM at saturating drug exposures. The data also demonstrated a statistically significant ( $p < 0.0001$ ) dose-dependent duration of downregulation of MCAM, or CD146, on Th17 cells.
- | In poster and e-poster sessions at the European Hematology Association's 21<sup>st</sup> Congress, presented new [quality of life data](#) for patients with AL amyloidosis. These studies included data on the relative burden of AL amyloidosis on health-related quality of life for patients, the impact of treatment-related symptoms on treatment compliance, impact on absenteeism, job productivity and loss, and validity of psychometric properties of the SF-36v2<sup>®</sup> Health Survey.

### Upcoming Research and Development Milestones

Prothena's pipeline includes four protein immunotherapy programs.

**NEOD001** is a monoclonal antibody for the potential treatment of AL amyloidosis:

- | Expect to complete enrollment in the Phase 3 VITAL Amyloidosis Study in the second quarter of 2017
- | Expect topline results from the Phase 2b PRONTO study in late 2017 - early 2018

**PRX002** is a monoclonal antibody for the potential treatment of Parkinson's disease and related synucleinopathies, and is the primary focus of Prothena's worldwide collaboration with Roche:

- | Expect topline results from Phase 1b multiple ascending dose study in patients with Parkinson's disease in the fourth quarter of 2016

**PRX003** is a monoclonal antibody for the potential treatment of inflammatory diseases, including psoriasis:

- | Expect to provide update on Phase 2 development strategy in September 2016
- | Expect interim data from the Phase 1b multiple ascending dose, proof-of-biology study in patients with psoriasis by mid-2017
- | Expect full topline results from the Phase 1b multiple ascending dose, proof-of-biology study in patients with psoriasis in the second half of 2017

**PRX004** is a monoclonal antibody for the potential treatment of TTR amyloidosis:

- | Expect to advance PRX004 into clinical development in late 2017 - early 2018

## **Second Quarter and First Six Months of 2016 Financial Results and Updated 2016 Financial Guidance**

Prothena reported a net loss of \$40.4 million and \$68.0 million for the second quarter and first six months of 2016, respectively, as compared to a net loss of \$18.3 million and \$33.5 million for the second quarter and first six months of 2015, respectively. Net loss per share for the second quarter and first six months of 2016 was \$1.18 and \$1.99, respectively, as compared to a net loss per share of \$0.59 and \$1.15 for the second quarter and first six months of 2015, respectively.

Prothena reported total revenue of \$0.3 million and \$0.6 million for the second quarter and first six months of 2016, respectively, as compared to total revenue of \$0.3 million and \$0.9 million for the second quarter and first six months of 2015, respectively. The decrease in revenue for the second quarter and first six months of 2016 was primarily due to lower revenue from Prothena's collaboration agreement with Roche.

Research and development (R&D) expenses totaled \$32.4 million and \$52.9 million for the second quarter and first six months of 2016, respectively, as compared to \$12.8 million and \$23.4 million for the second quarter and first six months of 2015, respectively. The increase in R&D expenses for the second quarter was primarily due to increased expenses for product manufacturing and personnel cost. The increase in R&D expense for the first six months was primarily due to increased expenses related to product manufacturing, clinical trials and personnel costs. R&D expenses included non-cash share-based compensation expense of \$1.8 million and \$3.2 million for the second quarter and first six months of 2016, respectively, as compared to \$1.0 million and \$1.7 million for the second quarter and first six months of 2015, respectively.

General and administrative (G&A) expenses totaled \$8.1 million and \$15.3 million for the second quarter and first six months of 2016, respectively, as compared to \$5.5 million and \$10.6 million for second quarter and first six months of 2015, respectively. The increase in G&A expenses for the second quarter and first six months was primarily due to increases in personnel costs. G&A expenses included non-cash share-based compensation expense of \$2.7 million and \$5.0 million in the second quarter and first six months of 2016, respectively, as compared to \$1.4 million and \$2.4 million in the second quarter and first six months of 2015, respectively.

Total non-cash share-based compensation expense was \$4.5 million and \$8.3 million for the second quarter and first six months of 2016, respectively, as compared to \$2.4 million and \$4.1 million for the second quarter and first six months of 2015, respectively.

As of June 30, 2016, Prothena had \$447.0 million in cash, cash equivalents and restricted cash and no debt.

As of July 22, 2016, Prothena had approximately 34.4 million ordinary shares outstanding.

The Company is updating its projected full year 2016 net cash burn from operating and investing activities, and expects it to be \$118 to \$128 million, representing an increase of approximately \$13 million, and expects to end the year with approximately \$376 million in cash, cash equivalents and restricted cash (midpoint). Based on the new clinical data from the NEOD001 Phase 1/2 study presented at ISA, the Company accelerated manufacturing expenses into the current fiscal year in order to increase its supply of NEOD001. Accelerating these manufacturing expenses into the 2016 operating year offers an opportunity to support NEOD001 investigator-sponsored trials, and to prepare to scale for potential commercial launch. The updated estimated full year 2016 net cash burn from operating and investing activities is primarily driven by an updated estimated net loss of \$144 to \$161 million, which includes an estimated \$18 million of non-cash share-based compensation expense.

## Upcoming Investor Conferences

Members of the senior management team will present and participate in investor meetings at the **2016 Webbush Pacgrow Healthcare Conference** on August 16, 2016 at 10:20 AM ET in New York, NY.

A live webcast of the presentation can be accessed through the investor relations section of the Company's website at [www.prothena.com](http://www.prothena.com). Following the live presentation, a replay of the webcast will be available on the Company's website for at least 90 days following the presentation date.

## About Prothena

Prothena Corporation plc is a global, late-stage clinical biotechnology company seeking to fundamentally change the course of progressive diseases with its clinical pipeline of novel therapeutic antibodies. Fueled by its deep scientific understanding built over decades of research in protein misfolding and cell adhesion - the root causes of many serious or currently untreatable amyloid and inflammatory diseases - Prothena has advanced several drug candidates into clinical trials while pursuing discovery of additional novel therapies. Our pipeline of antibody-based product candidates targets a number of potential indications including AL amyloidosis (NEOD001), Parkinson's disease and other related synucleinopathies (PRX002), inflammatory diseases, including psoriasis (PRX003), and TTR amyloidosis (PRX004).

## Forward-looking Statements

*This press release contains forward-looking statements. These statements relate to, among other things, the ability of our cash position to support advancement of multiple clinical development programs through key milestones; our confidence in the design and powering of the Phase 2b PRONTO and Phase 3 VITAL studies; the timing of completing enrollment in the Phase 3 VITAL study and reporting results from the Phase 2b PRONTO study for NEOD001; the timing of reporting results from the Phase 1b multiple ascending dose study for PRX002; the timing of providing an update on the Phase 2 development strategy and reporting interim data and full results from the Phase 1b multiple ascending dose study for PRX003; the timing of advancing PRX004 into clinical development; the opportunity to support NEOD001 investigator-sponsored trials and to prepare to scale for commercial launch; our anticipated net cash burn from operating and investing activities for 2016 and expected cash balance at the end of 2016; and our estimated net loss and non-cash share-based compensation expense for 2016. 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 25, 2016 and our subsequent Quarterly Reports on Form 10-Q filed with the SEC. 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 CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (unaudited - amounts in thousands except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Collaboration revenue	\$ 333	\$ 278	\$ 598	\$ 871
Total revenue	333	278	598	871
Operating expenses:				
Research and development	32,359	12,791	52,852	23,364
General and administrative	8,134	5,522	15,316	10,571
Total operating expenses	40,493	18,313	68,168	33,935

Loss from operations	<u>(40,160)</u>	<u>(18,035)</u>	<u>(67,570)</u>	<u>(33,064)</u>
Other expense	<u>(96)</u>	<u>(47)</u>	<u>(26)</u>	<u>46</u>
Loss before income taxes	<u>(40,256)</u>	<u>(18,082)</u>	<u>(67,596)</u>	<u>(33,018)</u>
Provision for income taxes	189	195	370	461
Net loss	<u>\$ (40,445)</u>	<u>\$ (18,277)</u>	<u>\$ (67,966)</u>	<u>\$ (33,479)</u>
Basic and diluted net loss per share	<u>\$ (1.18)</u>	<u>\$ (0.59)</u>	<u>\$ (1.99)</u>	<u>\$ (1.15)</u>
Shares used to compute basic and diluted net loss per share	34,358	30,792	34,192	29,106

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

	<b>June 30,</b>	<b>December 31,</b>
	<b>2016</b>	<b>2015</b>
<b>Assets</b>		
Cash and cash equivalents	\$ 442,948	\$ 370,586
Other current assets	<u>7,650</u>	<u>6,817</u>
Total current assets	450,598	377,403
Property and equipment, net	41,852	3,862
Restricted cash	4,056	—
Other assets	<u>6,056</u>	<u>3,971</u>
Total non-current assets	51,964	7,833
Total assets	<u><u>\$ 502,562</u></u>	<u><u>\$ 385,236</u></u>
<b>Liabilities and Shareholders' Equity</b>		
Accrued research and development	16,650	12,794
Other current liabilities	<u>16,806</u>	<u>9,422</u>
Total current liabilities	33,456	22,216
Non-current liabilities:	<u>38,241</u>	<u>2,351</u>
Total liabilities	71,697	24,567
Total shareholders' equity	<u>430,865</u>	<u>360,669</u>
Total liabilities and shareholders' equity	<u><u>\$ 502,562</u></u>	<u><u>\$ 385,236</u></u>

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