Sarepta Therapeutics and Clinigen Launch a Managed Access Program to Treat Patients with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

-- The MAP will be available to a limited number of patients who meet pre-specified medical criteria and can secure funding --

CAMBRIDGE, Mass., July 19, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a U.S. biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, and Clinigen Group plc's (AIM:CLIN, 'Clinigen') Idis Managed Access division, have initiated a Managed Access Program (MAP) for eteplirsen in certain geographies to treat eligible Duchenne muscular dystrophy (DMD) patients amenable to exon 51 skipping. This MAP (also known as an early / expanded access, or named patient program) provides a mechanism through which physicians can legally and ethically prescribe eteplirsen to patients who meet pre-specified medical criteria and where funding can be secured.

Initially, the program is being launched in select countries within Europe, North America and South America for certain patients where eteplirsen is not currently approved. Sarepta plans to expand the program to include more countries over time.

The program will be administered by Clinigen Group plc's Idis Managed Access division. Clinigen is the trusted global leader in access to unlicensed medicines delivering over 220 MAPs to thousands of patients, helping physicians access medicines when no other treatment options are available.

All requests must be submitted by the treating physician on behalf of the patient. Healthcare providers can obtain details about the EXONDYS 51® (eteplirsen) Managed Access Program by calling a Clinigen representative at +44 1283 494 340, or emailing medicine.access@clinigengroup.com.

For more information on the MAP, including the countries where the program is currently available to patients, please visit www.sarepta.com/community/managed-access-program.

About Eteplirsen

Eteplirsen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. Eteplirsen is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of eteplirsen in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of eteplirsen has not been established.

About Duchenne Muscular Dystrophy

DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 – 5,000 males worldwide. A devastating and incurable muscle-wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas of the body. The condition is universally fatal, and death usually occurs before the age of 30 generally due to respiratory or cardiac failure.

About Sarepta Therapeutics

Sarepta Therapeutics is a U.S. commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

About Clinigen Group

Clinigen Group plc (AIM:CLIN) is a global pharmaceutical and services company with a unique combination of businesses focused on providing access to medicines. Its mission is to deliver the right medicine to the right patient at the right time through three areas of global medicine supply; clinical trial, unlicensed and licensed medicines.

Forward-Looking Statement

This press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words

such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the MAP being a mechanism through which physicians can legally and ethically prescribe eteplirsen to eligible patients who meet pre-specified medical criteria and where funding can be secured; and Sarepta's plan to expand the Managed Access Program it initiated with Clinigen to include more countries over time.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta, in partnership with Clinigen, may not be able to establish and successfully conduct a Managed Access Program in one or more countries, and even if such program(s) are successfully conducted in each country targeted, Sarepta may not achieve any significant revenues from sales of eteplirsen under the MAP in one or more of the countries in which the MAP is launched; and Sarepta may not be able to commercialize eteplirsen in MAP countries even if the product has been available on a named patient basis. Any of the foregoing risks could adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2016 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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