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Clinigen signs agreement with Nippon Shinyaku for *Viltolarsen* Managed Access Program

Clinigen Group plc (AIM: CLIN, 'Clinigen'), the global pharmaceutical Services and Products company, announces it has signed an exclusive agreement with Nippon Shinyaku Co., Ltd ('Nippon Shinyaku') to implement a Managed Access Program for VILTEPSO® (*viltolarsen*) for patients with Duchenne muscular dystrophy (DMD) who are amenable to exon 53 skipping therapy.

DMD is a progressive muscle disease that primarily occurs in boys due to a genetic mutation in the dystrophin gene. This prevents the production of normal dystrophin resulting in reduced muscle strength/function that progressively continues to decline. There are many types of genetic mutations that can cause DMD. Viltolarsen skips exon 53; and therefore has the potential to rescue the dystrophin protein for 8–10% of DMD patients.[1]

Following finalization, the Managed Access Program will enable early access to VILTEPSO® for eligible patients in countries where it is not commercially available. VILTEPSO® is currently only approved in the US and Japan.

Under the terms of the agreement, Clinigen will manage key elements of the program including regulatory oversight, logistics and access management.

Pete Belden, Executive Vice President Services Division, Clinigen, said:

"We are pleased to partner with Nippon Shinyaku to enable access to a valuable new treatment option for DMD, a rare and progressive disease for which there remains a clear unmet medical need. This agreement underlines Clinigen's strength in partnering with biotechnology companies to provide services that enable quicker and broader access to new innovative therapies."

Kazuchika Takagaki, Director, Head of R&D, Nippon Shinyaku, said:

"We are delighted to help more DMD patients with critical unmet medical needs around the world on this important access program in partnership with Clinigen. We look forward to further contributing to the well-being of patients and their families."

Healthcare professionals can obtain details about the VILTEPSO® Program by calling the customer service team at +44 1932 824001 or emailing medicineaccess@clinigengroup.com.

Patients seeking medical information should contact their physician.

– Ends –

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Notes to Editors

About Clinigen Group

Clinigen Group plc (AIM: CLIN) is a global, specialist pharmaceutical services and products company focused on providing ethical access to medicines. Its' mission is to deliver the right medicine to the right patient at the right time. The Group operates from sites in North America, Europe, Africa and the Asia Pacific.

Clinigen has more than 1,000 employees across five continents in 14 countries, with supply and distribution hubs and operational centres of excellence in key long-term growth regions. The Group works with 34 of the top 50 pharmaceutical companies; interacting with over 20,000 healthcare professionals across more than 120 countries.

For more information on Clinigen, please visit http://www.clinigen.com

About Nippon Shinyaku

Nippon Shinyaku's mission is to help people lead healthier and happier lives. Through creating unique medicines that will bring hope to patients and families struggling with illness, we aim to be an organization trusted by the community. Please visit their website (http://www.nipponshinyaku.co.jp/english/) for products or detailed information.

Duchenne Muscular Dystrophy (DMD)

The incidence of DMD is reported as being 10.7 to 27.8 per 100,000 live male births worldwide.[2] DMD is a progressive form of muscular dystrophy that occurs primarily in males. DMD causes progressive weakness and loss of skeletal, cardiac, and pulmonary muscles. Early signs of DMD may include delayed ability to sit, stand or walk. There is a progressive loss of mobility, and by adolescence, patients with DMD may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications.

References

1. Komaki H, Takeshima Y, Matsumura T, Ozasa S, Funato M, Egawa Y, et al. (n.d.). A Japanese Phase I/II study of NS-065/ NCNP-01 (Viltolarsen), Exon 53 skipping drug, in patients with Duchenne muscular dystrophy—a dose-finding study. Mendoza: Poster presented at the 23rd International Annual Congress of the World Muscle Society; 2018.
2. Mah, J.K.; Korngut, L.; Dykeman, J.; Day, L.; Pringsheim, T.; Jette, N. A systematic review and meta-analysis on the epidemiology of Duchenne and Becker muscular dystrophy. Neuromuscul. Disord. 2014, 24, 482–491.