Following the recent news that Biogen's Spinraza (nusinersen) won approval from China's National Medical Products Association (NMPA) as a treatment for spinal muscular atrophy (SMA), Vinie Varkey, MSc, Senior Neurology & Ophthalmology Analyst at GlobalData, offers her view:

"The decision was not surprising given the recent efforts of NMPA to reform the process of approving drugs in China, a market that is characterized by growing demand for safe and effective medicines. In 2018, Spinraza was one of many drugs which was considered under a new priority review pathway by the Chinese Center for Drug Evaluation.

"Currently, Spinraza is the only approved drug for the treatment of patients with SMA, which severely affects the quality of life of patients especially if it is associated with an infant onset. Given Spinraza's first-to-market status, the therapy has been well received by SMA patients as well as their physicians in several countries such as the US and some of the European countries. However, the price tag associated with Spinraza has been a major factor in some countries, especially when it comes to reimbursement. For example, although Spinraza is approved for use in the UK, the National Institute for Health and Care Excellence does not recommend its use within the National Health Service network in England, citing lack of evidence on its cost-effectiveness. The situation is similar in Northern Ireland and Wales.

"While this represents an unfortunate situation for patients, their family and their physicians, real world data from countries in which the drug has been used and will be used is expected to play a supporting role in the decision making of other health institutes, which are yet to decide on recommending drugs for use in their country.

"The availability of such data for a rare disease such as SMA is also expected to help Biogen's standing against a strong pipeline of competitive drugs in late stage clinical development. Alternate mechanisms of actions such as a gene therapy to potentially cure the disease (Novartis' Zolgensma) or drugs that have a favorable route of administration (Novartis' branaplam and Roche's risdiplam) are some features of current pipeline drugs that excite patients as well as physicians.

"While the mechanisms of innovative therapies such as gene therapy are obvious, some conservatism from patients and physicians will be expected citing the lack of long-term data. And this is where Biogen will hope that long-term real world data will help the company to stand a good chance against alternate treatment options."