25 February 2019
EMA/832992/2018

Public summary of opinion on orphan designation

Human anti-promyostatin monoclonal antibody for the treatment of spinal muscular atrophy

On 14 December 2018, orphan designation (EU/3/18/2115) was granted by the European Commission to Yes Pharmaceutical Development Services GmbH, Germany, for human anti-promyostatin monoclonal antibody (also known as SRK-015) for the treatment of spinal muscular atrophy.

What is spinal muscular atrophy?

Spinal muscular atrophy is an inherited disease usually diagnosed in the first year of life that affects the motor neurons (nerves from the brain and spinal cord that control muscle movements). Patients with the disease lack a protein called 'survival motor neuron' (SMN), which is essential for the normal functioning and survival of motor neurons. Without this protein, the motor neurons deteriorate and eventually die. This causes the muscles to fall into disuse, leading to muscle wasting (atrophy) and weakness.

Spinal muscular atrophy is a long-term debilitating and life-threatening disease because it causes breathing problems and muscle wasting that worsens over time.

What is the estimated number of patients affected by the condition?

At the time of designation, spinal muscular atrophy affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 21,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, one medicine, Spinraza, was authorised for the treatment of spinal muscular atrophy. Patients also received supportive treatment to help them and their families cope with the symptoms of the disease. This included chest physiotherapy and physical aids to support muscle function, and ventilators to help with breathing.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with spinal muscular atrophy. Early laboratory data indicate that the medicine may improve muscle mass and muscle strength in patients who cannot be treated with the authorised medicine.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise, attach to and block a protein called promyostatin. Promyostatin is the inactive form of myostatin, a protein present in muscle cells that prevents muscle growth and regeneration. By blocking promyostatin, the medicine is expected to increase muscle mass and strength in patients with spinal muscular atrophy.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with spinal muscular atrophy had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for spinal muscular atrophy. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 recommending the granting of this designation.

_____

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Human anti-promyostatin monoclonal antibody	Treatment of spinal muscular atrophy
Bulgarian	Човешко анти-промиостатиново моноклонално антитяло	Лечение на спинална мускулна атрофия
Croatian	Ljudsko protu-promiostatin monoklonsko protutijelo	Liječenje spinalne mišićne atrofije
Czech	Humánní anti-promyostatinová monoklonální protilátka	Léčba spinální muskulární atrofie
Danish	Humant anti-promyostatin monoklonalt antistof	Behandling af spinal muskelatrofi
Dutch	Humaan anti-promyostatine monoklonaal antilichaam	Behandeling van spinale spieratrofie
Estonian	Inimese anti-promüostatiini monoklonaalne antikeha	Spinaalse lihasatroofia ravi
Finnish	Ihmisen monoklonaalinen anti-promyostatiinivasta-aine	Spinaalisen lihasatrofian hoito
French	Anticorps monoclonal humain anti-promyostatine	Traitement de l'amyotrophie spinale
German	Humaner monoklonaler anti-Promyostatin-Antikörper	Behandlung der spinalen Muskelatrophie
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα έναντι της προμυοστατίνης	Θεραπεία της νωτιαίας μυϊκής ατροφίας
Hungarian	Humán anti-promyostatin monoklonális antitest	Spinális izomatrophia kezelése
Italian	Anticorpo monoclonale umano anti-promiostatina	Trattamento dell'atrofia muscolare spinale
Latvian	Cilvēka anti-promiostatīna monoklonālā antiviela	Spinālās muskuļu atrofijas ārstēšana
Lithuanian	Žmogaus anti-promiostatino monokloninis antikūnas	Spinalinės raumenų atrofijos gydymas
Maltese	Antikorp monoklonali uman kontra l-promyostatin	Kura tal-atrofija muskolari tas-sinsla
Polish	Ludzkie przeciwciało monoklonalne przeciw promiostatynie	Leczenie rdzeniowego zaniku mięśni
Portuguese	Anticorpo monoclonal humano anti-promiostatina	Tratamento da atrofia muscular espinal
Romanian	Anticorp monoclonal uman anti-promiostatină	Tratamentul amiotrofiei spinale
Slovak	Ľudská anti-promyostatínová monoklonálna protilátka	Liečba spinálnej svalovej atrofie
Slovenian	Humano monoklonsko protitelo proti promiostatinu	Zdravljenje spinalne mišične atrofije

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Anticuerpo monoclonal humano anti-promiostatina	Tratamiento de la atrofia muscular espinal
Swedish	Human anti-promyostatin monoklonal antikropp	Behandling av spinal muskelatrofi
Norwegian	Humant anti-promyostatin monoklonalt antistoff	Behandling av spinal muskelatrofi
Icelandic	Einstofna mannamótefni gegn pro-myostatin	Meðferð við mænuvöðvarýrnunar