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Public summary of opinion on orphan designation

Autologous human bone marrow-derived haematopoietic and mesenchymal stem cells depleted of erythrocytes, monocytes and lymphocytes for the treatment of spinal cord injury

On 24 April 2019, orphan designation (EU/3/19/2153) was granted by the European Commission to Neuroplast B.V., Netherlands, for autologous human bone marrow-derived haematopoietic and mesenchymal stem cells depleted of erythrocytes, monocytes and lymphocytes (also known as Neurocell) for the treatment of spinal cord injury.

What is spinal cord injury?

The spinal cord can be injured through accidents or by internal causes such as tumours or bleeding within the spine putting pressure on the spinal cord. The resulting damage to the nerves that run through the cord and that branch out from it can stop the flow of nerve impulses between the brain and the body. This leads to loss of feeling, paralysis and even death, depending on the severity of the injury and where it is located. Patients may also experience loss of control of bladder or bowels, breathing difficulties, blood clots in veins and lungs, and recurring infections.

Spinal cord injury is debilitating and life threatening because it can cause paralysis of the arms and legs and reduces life expectancy.

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

At the time of designation, spinal cord injury affected approximately 4.46 in 10,000 people in the European Union (EU). This was equivalent to a total of around 231,000 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).

[*]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 518,400,000 (Eurostat 2019).

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What treatments are available?

At the time of designation, methylprednisolone (a steroid) was authorised for the treatment of spinal cord injury in some countries in the EU. Methylprednisolone reduces the inflammation and pressure on the spinal cord that can occur soon after it is damaged. Patients with spinal cord injury could also have decompression surgery to reduce the pressure on the spine. Other medicines or procedures were used to manage various symptoms such as spasticity (painful muscle spasm) and incontinence.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with spinal cord injury. Laboratory studies suggest that the medicine may improve the patient's ability to move more than the authorised treatment. 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 made of stem cells taken from the bone marrow of the patient. Stem cells can develop into different types of cells. The exact way these stem cells work is not clear but when injected back into the patient's spine, they are expected to help repair the damaged nerve cells, improving the symptoms of patients with spinal cord injury.

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 cord injury had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of spinal cord injury or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 21 March 2019, 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 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	Autologous human bone marrow-derived haematopoietic and mesenchymal stem cells depleted of erythrocytes, monocytes and lymphocytes	Treatment of spinal cord injury
Bulgarian	Хемопоетични и мезенхимни стволови клетки без еритроцити, моноцити и лимфоцити, получени от автоложен човешки костен мозък	Лечение на гръбначно-мозъчни травми
Croatian	Autologne krvotvorne i mezenhimske matične stanice iz ljudske koštane srži sa smanjenim brojem eritrocita, monocita i limfocita	Liječenje ozljede kralježnične moždine
Czech	Autologní lidské hematopoetické a mezenchymální kmenové buňky derivované z kostní dřeně s deplecí erytrocytů, monocytů a lymfocytů	Léčba míšního traumatu
Danish	Autologe humane knoglemarvsafledte hæmatopoietiske og mesenkymale stamceller depleteret for erytrocytter, monocytter og lymfocytter	Behandling af rygmarvslæsion
Dutch	Autologe uit humaan beenmerg afkomstige hematopoietische en mesenchymale stamcellen, ontdaan van erytrocyten, monocyten en lymfocyten	Behandeling van ruggenmergletsel
Estonian	Erütrotsüütide, monotsüütide ja lümfotsüütideta autoloogsed inimese luuüdist saadud hematopoeetilised ja mesenhümaalsed tüvirakud.	Seljaaju vigastuse ravi
Finnish	Autologiset ihmisen luuydinperäiset hematopoieettiset ja mesenkymaaliset kantasolut, jotka eivät sisällä erytrosyyttejä, monosyyttejä ja lymfosyyttejä.	Selkäydinvamman hoito
French	Cellules souches hématopoïétiques et mésenchymateuses dérivées de moelle osseuse humaine autologues débarrassées des érythrocytes, monocytes et lymphocytes	Traitement des lesions de la moëlle épinière

[1] At the time of designation

Language	Active ingredient	Indication
German	Autologe, aus humanem Knochenmark gewonnene hämatopoetische und mesenchymale Stammzellen nach Depletion von Erythrozyten, Monozyten und Lymphozyten	Behandlung der Rückenmarkverletzung
Greek	Αυτόλογα αιμοποιητικά και μεσεγχυματικά βλαστοκύτταρα από ανθρώπινο μυελό των οστών απαλλαγμένα από ερυθροκύτταρα, μονοκύτταρα και λεμφοκύτταρα	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Vörösvérsejtektől, monocitáktól és limfocitáktól mentesített autológ, human, csontvelő eredetű hemopoetikus és mesenchimalis őssejtek.	Gerincvelő sérülés kezelése
Italian	Cellule staminali ematopoietiche e mesenchimali derivate dal midollo osseo umano autologo prive di eritrociti, monociti e linfociti	Trattamento delle lesioni del midollo spinale
Latvian	No kaulu smadzenēm iegūtas autologas cilvēka hematopoētiskās un mezenhimālās cilmes šūnas, kas attīrītas no eritrocītiem, monocītiem un limfocītiem	Muguras smadzeņu traumu ārstēšana
Lithuanian	Autologinės iš žmogaus kaulų čiulpų išskirtos hematopoetinės ir mezenchiminės kamieninės ląstelės, atskirtos nuo eritrocitų, monocitų ir limfocitų	Nugaros smegenų sužalojimo gydymas
Maltese	Ċelloli staminali ematopojetiċi u mesenkimali awtologi derivati mill-mudullun tal-għadam tal-bniedem imnaqqsa minn eritroċiti, monoċiti u limfoċiti	Kura ta’ korriment tan-nerv qawwi li jgħaddi minn ġos-sinsla
Polish	Autologiczne ludzkie hematopoetyczne i mezenchymalne komórki macierzyste pozyskane ze szpiku kostnego, pozbawione erytrocytów, monocytów i limfocytów.	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Células estaminais hematopoiéticas e mesenquimais autólogas derivadas de medula óssea humana, e livres de eritrócitos, monócitos e linfócitos	Tratamento da lesão da medula espinal
Romanian	Celule stem hematopoietice și mezenchimale derivate din măduvă osoasă umană autologă, cu eritrocite, monocite și limfocite îndepărtate	Tratamentul leziunilor măduvei spinării
Slovak	Autológne hematopoetické a mezenchymálne kmeňové bunky odvodené od erytrocytov, monocytov a lymfocytov z ľudskej kostnej drene	Liečba poškodenia miechy

Language	Active ingredient	Indication
Slovenian	Avtologne hematopoetske in mezenhimske matične celice, pridobljene iz človeškega kostnega mozga, z odstranjenimi eritrociti, monociti in limfociti	Zdravljenje poškodbe hrbtenjače
Spanish	Células madre hematopoyéticas y mesenquimales para trasplante derivadas de la médula ósea desprovista de eritrocitos, monocitos y linfocitos	Tratamiento de las lesiones de la médula espinal
Swedish	Autologa mänskliga hematopoetiska och mesenkymala stamceller som härrör från benmärg, utarmade på erytrocyter, monocyter och lymfocyter	Behandling av ryggmärgsskada
Norwegian	Autologe humane hematopoietiske og mesenkymale stamceller fra benmarg depletert for erytrocytter, monocytter og lymfocytter	Behandling av ryggmargsskade
Icelandic	Samgena, manna, blóðmyndandi- og bandvefsstofnfrumur úr beinmerg sem eru rauðkorna-, einkjörnunga- og eitilfrumusnauðar	Meðferð mænuskaða vegna slyss