10 May 2017
EMA/206895/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous adult bone marrow-derived non-expanded CD133+ haematopoietic stem cells for the treatment of Asherman's syndrome

On 20 April 2017, orphan designation (EU/3/17/1862) was granted by the European Commission to Igenomix, S.L., Spain, for autologous adult bone marrow-derived non-expanded CD133+ haematopoietic stem cells for the treatment of Asherman's syndrome.

What is Asherman's syndrome?

Asherman's syndrome is a condition caused by scarring and sticking together (adhesions) of tissues in the lining and neck of the womb (the endometrium and cervix), often following surgery or the use of an intrauterine device. Adhesions may occur only in a small part of the womb, or be more extensive and severe, causing the front and back walls of the womb to stick to one another. Women with Asherman's syndrome have pain, menstrual cycle (period) irregularities such as reduced or absent menstrual bleeding, frequent miscarriages and infertility.

Asherman's syndrome is a debilitating condition due to the pain, miscarriages and infertility women with the condition experience, and because of the psychological distress associated with it.

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

At the time of designation, Asherman's syndrome affected approximately 4.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 227,000 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, treatments for Asherman's syndrome included surgery to remove scar tissue and adhesions, use of devices to prevent the recurrence of adhesions, and hormonal treatment to restore the function of the endometrium.

*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 515,700,000 (Eurostat 2017).

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 Asherman's syndrome because early studies showed that it could improve pregnancy rates in patients affected by the condition in whom other treatments had failed. 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 up of the patient's own stem cells. Stem cells are cells that can develop into different types of cell. To make the medicine, a type of stem cells called CD133+ stem cells are collected from the bone marrow until a sufficient number is available.

Although it is not fully understood how they work, when these cells are injected into the blood vessels of the womb they are expected to stimulate the growth of blood vessels in the endometrium. This is expected to facilitate scar healing, thus improving the symptoms of the condition. These stem cells may also be able to develop into cells of the endometrium, thus helping to regenerate the damaged tissue in women with the condition.

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, clinical trials with the medicine in patients with Asherman's syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for Asherman's syndrome or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 March 2017 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, on the medicine's rare disease designations page.

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 adult bone marrow-derived non-expanded CD133+ haematopoietic stem cells	Treatment of Asherman's syndrome
Bulgarian	Автоложни некултивирани CD133+ хематопоетични стволови клетки, получени от костен мозък на възрастен	Лечение на синдром на Ашерман
Croatian	Autologne ne-ekspandirane CD133+ hematopoetske matične stanice derivirane iz koštane srži odraslih	Liječenje Ashermanovog sindroma
Czech	Autologní hematopoetické neexpandované CD 133 + dospělé buňky kostní dřeně	Léčba Ashermanova syndrome
Danish	Autologe knoglemarvsderiverede ikke-opformerede CD133+ hæmatopoietiske stamceller	Behandling af Ashermans syndrom
Dutch	Uit adult beenmerg verkregen autologe niet-geëxpandeerde CD133+ haematopoiëtische stamcellen	Behandeling van Asherman's syndroom
Estonian	Autoloogsed täiskasvanu luuüdist lähtunud mittelaiendatud CD133+ hematopoeetilised tüvirakud	Ashermanni sündroomi ravi
Finnish	Autologiset aikuisen ihmisen luuytimestä peräisin olevat ei-laajennetut CD133+ hematopoieettiset solut	Ashermanin syndrooman hoito
French	Cellules souche autologue hématopoïétiques adulte issues de la moelle osseuse, non-prolifératives CD133+	Traitement du Syndrome d'Asherman
German	Autologe, adulte, aus dem Knochenmark stammende, unexpandierte CD133+ hämatopoetische Stammzellen	Behandlung des Asherman Syndroms
Greek	Αυτόλογα μη-πολλαπλασιασμένα CD133+ αιμοποιητικά βλαστικά κύτταρα από μυελό οστών ενηλίκου	Θεραπεία του συνδρόμου Asherman
Hungarian	Autológ felnőtt csontvelőből származó nem expandált CD133+ hematopoietikus őssejtek	Asherman szindróma kezelése
Italian	Cellule autologhe ematopoietiche staminali CD133+ non espanse derivate da midollo osseo adulto	Trattamento della Sindrome di Asherman
Latvian	Autologas, nepavairotas pieaugušo CD133+ hematopoētiskās cilmes šūnas, kas iegūtas no kaula smadzenēm	Ašermaņa (*Asherman*) sindroma ārstēšana
Lithuanian	Autologinės iš suaugusiojo kaulų išskirtos nepagausintos CD133+ hemapoetinės kamieninės ląstelės	*Asherman* sindromo gydymas
Maltese	Mudullun awtologu tal-adult ġej minn ċelloli staminali ħaematopoietikli CD133+ li ma jespandux	Kura tas-sindromu ta' Asherman
Polish	Autologiczne nie namnożone komórki krwiotwórcze CD133+ wywiedzione z dorosłego szpiku kostnego	Leczenie zespołu Ashermana

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Células estaminais hematopoiéticas CD133+ autólogas não expandidas derivadas de medula óssea adulta	Tratamento da síndrome de Asherman
Romanian	Celule stem adulte autologe hematopoietice CD133+ non-expandate derivate din maduva osoasa adulta	Tratamentul sindromului Asherman
Slovak	Autológne neexpandované CD 133+ hematopoietické kmeňové bunky z dospelej kostnej drene	Liečba Ashermanovho syndrómu
Slovenian	Avtologne krvotvorne neekspandirane CD133+ matične celice pridobljene iz odraslega kostnega mozga mobilizirane iz periferne krvi	Zdravljenje Ashermanovega sindroma
Spanish	Células madre adultas hematopoiéticas autologas de médula ósea no expandidas CD133+ mobilizadas a partir de sangre periférica	Tratamiento del sindrome de Asherman
Swedish	Vuxna autologa benmärgsderivereade icke-expanderade CD133+ haematopoetiska stamceller	Behandling av Ashermans syndrome
Norwegian	Autologe, voksne, beinmargsderiverte, ikke-ekspanderte CD133+ hematopoetiske stamceller	Behandling av Ashermans syndrom
Icelandic	Samgena ekki útvíkkaðar blóðmyndandi CD133+ stofnfrumur úr beinmerg	Meðferð við Asherman heilkenni