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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lipopolysaccharide of *Ochrobactrum intermedium* for the prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 11 January 2012, orphan designation (EU/3/11/941) was granted by the European Commission to Diomune, S.L., Spain, for lipopolysaccharide of *Ochrobactrum intermedium* for the prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age.

What is sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age?

Sepsis is a severe bacterial infection of the blood, which may occur in newborn babies. Premature babies born eight weeks or more too early (32 weeks of gestational age or less) have a higher risk of sepsis than other babies. This is because their immune system, skin and moist body surfaces are not yet fully developed, and because they need invasive procedures such as mechanical ventilation (using a machine to help them to breathe) or administration of medicines or feeding through a vein, which can put them at risk of infections in neonatal intensive care units.

Sepsis is a life-threatening condition which may cause multiple organ failure. Sepsis is one of the most common causes of death in premature babies born at 32 weeks of gestational age or less.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of premature babies of less than or equal to 32 weeks of gestational age at risk of sepsis was estimated to be approximately 1.5 in 10,000 in the European



Union (EU). This was equivalent to a total of around 76,000 babies*, 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 methods of prevention are available?

At the time of designation, several antibiotics were used to treat and to prevent sepsis in premature babies.

The sponsor has provided sufficient information to show that lipopolysaccharide of *Ochrobactrum intermedium* might be of significant benefit for premature babies at risk of sepsis because it works in a different way to existing treatments and may improve the outcome of patients at risk of this condition. 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?

The medicine is expected to mildly stimulate the immune system (the body's natural defences) of premature infants because it is very similar to a structure on the surface of bacteria, which may cause sepsis. This structure is recognised as 'foreign' by immune cells because it attaches to and activates two receptors called 'Toll-like receptor 2' and 'Toll-like receptor 4' on the surface of these immune cells. Their activation is expected to stimulate other immune cells to attack bacteria when the premature infant is exposed to them. This is expected to help prevent sepsis.

What is the stage of development of this medicine?

The effects of lipopolysaccharide of *Ochrobactrum intermedium* 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 at risk of sepsis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the prevention of sepsis 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 9 November 2011 recommending the granting of this designation.

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Lipopolysaccharide of <i>Ochrobactrum intermedium</i>	Prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age
Bulgarian	Липополизахарид на <i>Ochrobactrum intermedium</i>	Превенция на сепсис, при рискови недоносени новородени, с гестационна възраст, по-малка или равна на 32 седмици
Czech	Lipopolysaccharid <i>Ochrobactrum intermedium</i>	Prevence pozdní sepse u kojenců narozených v 32. týdnu těhotenství či dříve
Danish	Lipopolysaccharid af <i>Ochrobactrum intermedium</i>	Forebyggelse af sepsis hos præmature risiko-spædbørn født før eller i 32. gestationsuge
Dutch	Lipopolysaccharide van <i>Ochrobactrum intermedium</i>	Preventie van sepsis bij risicovolle premature zuigelingen met een zwangerschapsleeftijd van minder of gelijk aan 32 weken.
Estonian	<i>Ochrobactrum intermedium</i> lipopolüsahhariid	Sepsise ennetamine kõrgenenud riskiga 32. või väiksema rasedusnädalaga enneaegsetel imikutel
Finnish	<i>Ochrobactrum intermedium</i> in lipopolysakkaridi	Keskosten, joiden sikiöikä ("gestational age") on enintään 32 viikkoa ja jotka kuuluvat sepsiksen riskiryhmään, sepsiksen ehkäisy
French	Lipopolysaccharide d' <i>Ochrobactrum intermedium</i>	Prévention de sepsis chez les nouveau-nés prématurés de 32 semaines ou moins d'âge gestationnel
German	Lipopolysaccharide aus <i>Ochrobactrum intermedium</i>	Prävention einer Sepsis bei gefährdeten Frühgeborenen mit einem Gestationsalter von bis zu 32 Wochen
Greek	Λιποπολυσακχαρίτης του <i>Ochrobactrum intermedium</i>	Πρόληψη της σήψης σε πρόωρα νεογνά που βρίσκονται σε κίνδυνο, με ηλικία κύησης μικρότερης ή ίσης των 32 εβδομάδων .
Hungarian	<i>Ochrobactrum intermedium</i> lipopoliszacharid	32 vagy annál rövidebb gesztációs idejű koraszülöttek szepszisének megelőzése
Italian	Lipopolisaccaride di <i>Ochrobactrum intermedium</i>	Prevenzione della sepsi nei neonati prematuri di ≤ 32 settimane d'età gestazionale
Latvian	<i>Ochrobactrum intermedium</i> lipopolisaharīds	Sepses profilaksei augsta riska jaundzimušiem ar gestācijas vecumu mazāku vai vienādu ar 32 nedēļām
Lithuanian	<i>Ochrobactrum intermedium</i> lipopolisacharidas	Neišnešiotų, 32 ir mažiau gestacijos savaitių amžiaus, naujagimių, esančių rizikos grupėje, sepsio prevencija
Maltese	Lipopolysaccharide ta' <i>Ochrobactrum intermedium</i>	Prevenzjoni ta' sepsis fi trabi prematuri fil-periklu li jkollhom età inqas minn jew sa 32 ġimgħa ta' tqala

¹ At the time of designation

Language	Active ingredient	Indication
Polish	Lipopolisacharyd <i>Ochrobactrum Intermedium</i>	Zapobieganie sepsy u wcześniaków urodzonych przed lub w 32 tygodniu ciąży
Portuguese	Lipopolissacarídeo de <i>Ochrobactrum intermedium</i>	Prevenção de sépsis de início tardio em prematuros com 32 semanas ou menos, de idade gestacional
Romanian	Lipopolizaharida bacilului <i>Ochrobactrum intermedium</i>	Profilaxia sepsisului la nou-născuții prematur aflați la risc cu vârsta gestațională mai mică sau egală cu 32 săptămâni
Slovak	Lipopolysacharid <i>Ochrobactrum intermedium</i>	Prevenca sepsy u rizikových predčasne narodených detí s gestačným vekom 32 týždňov alebo menej
Slovenian	Lipopolisaharid od <i>Ochrobactrum intermedium</i>	Preprečevanje sepse pri novorojenčkih gestacijske starosti 32 tednov ali manj
Spanish	Lipopolisacárido de <i>Ochrobactrum intermedium</i>	Prevención de la sepsis en neonatos prematuros en riesgo y con edad gestacional igual o menor a 32 semanas
Swedish	Lipopolysackarider <i>Ochrobactrum intermedium</i>	Förebyggande av sepsis hos prematura spädbarn i riskzonen, födda före eller i gestationsvecka 32
Norwegian	Lipopolysakkarid av <i>Ochrobactrum intermedium</i>	Forebygging av sepsis hos premature spedbarn med gestasjonsalder mindre enn eller lik 32 uker som har økt risiko
Icelandic	Lípópólýsaccharíð af <i>Ochrobactrum intermedium</i>	Vörn gegn sýklasóttar hjá fyrirburum eftir ≤ 32 vikna meðgöngu við fæðingu