17 April 2015
EMA/COMP/29801/2014 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mixture of recombinant human IgG1 monoclonal antibodies against human cytomegalovirus envelope glycoproteins for the prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection

First publication	31 March 2014
Rev.1: withdrawal from the Community Register	17 April 2015

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.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2015 on request of the Sponsor.

On 19 February 2014, orphan designation (EU/3/14/1235) was granted by the European Commission to Roche Registration Limited, United Kingdom, for mixture of recombinant human IgG1 monoclonal antibodies against human cytomegalovirus envelope glycoproteins for the prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection.

What is congenital cytomegalovirus infection following primary cytomegalovirus infection?

Cytomegalovirus is a common virus that usually only causes mild infection, with symptoms such as fever and sore throat. Most people get infected at some stage during their lifetime but are very often unaware of it. After infection, the virus usually remains in the body in a 'latent' (inactive) state and only becomes active if the body's immune system (its natural defences) is weakened. Active cytomegalovirus disease can affect several organs in the body, such as the eyes, liver, lungs and the gastrointestinal tract (the stomach and intestines) and cause organ failure. If a woman is infected for the first time (primary infection) with cytomegalovirus while pregnant, the virus can pass to the unborn baby (congenital infection) as it does not yet have a very strong immune system. Congenital

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infection can cause severe disease at birth and lead to growth retardation and brain damage. Congenital cytomegalovirus disease is long-term debilitating and life-threatening.

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

At the time of designation, the number of patients at risk of congenital cytomegalovirus infection following primary cytomegalovirus infection was less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 methods of prevention are available?

At the time the application was submitted there were no medicines authorised in the EU for the prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection in pregnant women.

How is this medicine expected to work?

The medicine contains a mixture of two antibodies (proteins) that recognise and attach to specific structures on the surface of cytomegalovirus. These structures are part of the mechanism that the virus uses to enter and infect the cells of the body. By attaching to and blocking these structures, the medicine is expected to help prevent the virus from infecting the developing baby, and so prevent congenital cytomegalovirus infection and its consequences.

What is the stage of development of this medicine?

The effects of this 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 pregnant women with primary cytomegalovirus infection had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection in pregnant women. Orphan designation of the medicine had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2014 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 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mixture of recombinant human IgG1 monoclonal antibodies against human cytomegalovirus envelope glycoproteins	Prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection
Bulgarian	Смес от рекомбинантни човешки IgG1 моноклонални антитела, срещу енвелопните гликопротеини на човешкия цитомегаловирус.	Превенция на вродена инфекция с цитомегаловирус след първична цитомегаловирусна инфекция.
Czech	Směs monoklonální protilátky rekombinantního lidského IgG1 směrovaná na obalový glykoprotein lidského cytomegaloviru	Prevence kongenitální cytomegalovirové infekce po primární cytomegalovirové infekci
Croatian	Mješavina rekombinantnih ljudskih IgG1 monoklonskih protutijela protiv glikoproteina ovojnice ljudskog citomegalovirusa	Prevencija kongenitalne infekcije citomegalovirusom nakon primarne infekcije citomegalovirusom
Danish	Blanding af rekombinante human IgG1 monoclonale antistoffer rettet mod humane cytomegalovirus kappe-glycoproteiner	Forebyggelse af kongenit cytomegalovirus-infektion efter førstegangs cytomegalovirus-infektion
Dutch	Mengeling van recombinant humaan IgG1 monoclonale antilichamen gericht tegen humaan cytomegalovirus envelope glycoproteïne	Preventie van congenitale cytomegalovirus-infectie na primaire cytomegalovirus-infectie
Estonian	Mikstuur rekombinantse inimese IgG1 monoklonaalsetest anikehadest inimese tsütomegaloviiruse ümbrik glükoproteiinide vastu	Kaasasündinud tsütomegaloviirus-infektsiooni ennetamine peale esmast tsütomegaloviirusinfektsiooni
Finnish	Rekombinanttitekniikalla tehtyjen ihmisen monoklonaalisten, ihmisen sytomegaloviruksen vaipan glykoproteiinien IgG1-vasta-aineiden seos	Primaarin sytomegalovirustulehduksen aiheuttaman synnynnäisen sytomegalotulehduksen ehkäisy
French	Mélange d'anticorps monoclonaux recombinants humains IgG1 contre les glycoprotéines de l'enveloppe à cytomégalovirus humain	Prévention de l'infection congénitale à cytomégalovirus à la suite d'une primo-infection par le cytomégalovirus
German	Mischung rekombinanter menschlicher monoklonaler IgG1-Antikörper gegen Hüllglykoproteine des menschlichen Cytomegalovirus	Prävention der angeborenen Zytomegalie-Virus-Infektion nach primärer Zytomegalie-Virus-Infektion
Greek	Μίγμα ανασυνδυασμένων ανθρώπινων IgG1 μονοκλωνικών αντισωμάτων έναντι γλυκοπροτεϊνών του φακέλου του κυτταρομεγαλοϊού του ανθρώπου	Πρόληψη της εκ γενετής λοίμωξης με μεγαλοκυτταροϊό μετά από πρωτοπαθή λοίμωξη με μεγαλοκυτταροϊό

[1] At the time of designation

Language	Active ingredient	Indication
Hungarian	Humán IgG1 monoklonális antitest humán cytomegalovirus glikoprotein burok ellen	Primer cytomegalovirus-fertőzés okozta congenitalis cytomegalovirus-fertőzések megelőzése
Italian	Miscela di anticorpi umani ricombinanti monoclonali IgG1 contro glicoproteine del citomegalovirus umano	Prevenzione dell'infezione congenita da citomegalovirus a seguito di un'infezione primaria da citomegalovirus
Latvian	Pret citamegalovīrusa āķējā apvalka glikoproteīniem vērstu rekombinantu cilvēka IgG1 monoklonālo antivielu maisījums	Iedzimtas citomegalovīrusu infekcijas profilakse pēc primāras citomegalovīrusu infekcijas
Lithuanian	Rekombinantinio žmogaus IgG1 monokloninių antikūnų prieš žmogaus citomegaloviruso apvalkalo glikoproteinus mišinys	Įgimtos citomegaloviruso infekcijos, sąlygotos pirminės citomegaloviruso infekcijos, prevencija
Maltese	Taħlita ta' antikorpi monoklonali IgG1 umani rikombinanti kontra l-glikoproteini tal-involukru taċ-ċitomegalovirus uman	Prevenzjoni ta' infezzjoni konġenitali minn ċitomegalovirus wara infezzjoni primarja kkawżata minn ċitomegalovirus
Polish	Mieszanina rekombinowanych ludzkich przeciwciał monoklonalnych IgG1 przeciw glikoproteinom otoczki ludzkiego wirusa cytomegalii	Zapobieganie wrodzonym zakażeniom wirusem cytomegalii spowodowanym pierwotnym zakażeniem wirusem cytomegalii
Portuguese	Mistura de anticorpos monoclonais recombinantes humanos de tipo IgG1 anti glicoproteínas do envelope do citomegalovírus humano	Prevenção de infecção congénita pelo citomegalovírus a seguir à infecção primária pelo citomegalovírus
Romanian	Amestec de anticorpi monoclonali IgG1 umani recombinanţi anti glicoproteinele anvelopei citomegalovirusului uman	Prevenirea apariţiei infecţiei congenitale cu citomegalovirus după infecţia primară cu citomegalovirus
Slovak	Zmes rekombinantných ľudských IgG1 monoklonánych protilátok proti glykoproteínom obálky ľudského cytomegalovírusu	Prevencia vrodenej cytomegalovírusovej infekcie po primárnej cytomegalovírusovej infekcii
Slovenian	Mešanica rekombinantnih humanih monoklonskih IGG1 protiteles proti proteinom ovojnice humanega glikoproteina	Preprečevanje kongenitalne okužbe s citomegalovirusom po primarni okužbi s citomegalovirusom
Spanish	Mezcla de anticorpos monoclonales recombinantes humanos de tipo IgG1 anti glicoproteínas do envelope do citomegalovírus humano	Prevención de la infección congénita por citomegalovirus tras infección primaria por citomegalovirus
Swedish	Blandning av rekombinanta mänskliga IgG1 monoklonala antikroppar riktade mot glykoproteiner i höljet av mänskligt cytomegalovirus	Förebyggande av kongenital cytomegalovirusinfektion efter primär cytomegalovirusinfektion

Language	Active ingredient	Indication
Norwegian	Blanding av rekombinante humane IgG1 monoklonale antistoffer mot humant cytomegalovirus kappeglykoproteiner	Forebygging av medfødt cytomegalovirusinfeksjon etter primær cytomegalovirusinfeksjon
Icelandic	Blanda af raðbrigða manna IgG1 einstofna mótefnum gegn manna cýtómegalóveiru hjúp próteinum	Til forvarnar gegn meðfæddri stórfrumuveirusýkingu í kjölfar fyrstu stórfrumuveirusýkingar hjá barnshafandi konum