6 May 2015
EMA/COMP/127039/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

5′-$A_SC_SA_ST_SC_SA_SG_ST_SC_ST_SG_SA_SU_SA_SA_SG_SC_ST_SA$-3′ for the treatment of Alport syndrome

On 19 March 2015, orphan designation (EU/3/15/1451) was granted by the European Commission to CTI Clinical Trial and Consulting Services Europe GmbH, Germany, for 5′-$A_SC_SA_ST_SC_SA_SG_ST_SC_ST_SG_SA_SU_SA_SA_SG_SC_ST_SA$-3′ (also known as RG-012) for the treatment of Alport syndrome.

What is Alport syndrome?

Alport syndrome is an inherited condition caused by a mutation (defect) in one of a group of genes responsible for producing type IV collagen, a fibrous protein needed to form the membranes that separate and support cells in organs such as the kidney, ear and eye. In patients with Alport syndrome, these membranes have an abnormal structure, so the organs cannot develop and function properly. Patients therefore experience internal scarring of the kidney and gradually worsening kidney function that eventually results in kidney failure. Patients also suffer hearing loss and may develop cataracts and visual impairment.

Alport syndrome is a long-term debilitating disease due to the progressive kidney damage and impaired hearing and vision; it is potentially life threatening because it results in kidney failure that requires dialysis or transplantation.

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

At the time of designation, Alport syndrome affected not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 103,000 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 512,900,000 (Eurostat 2015).

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What treatments are available?

At the time of designation, no satisfactory methods of treatment were authorised in the EU for patients affected by the condition. Patients were given medicines that act on the renin-angiotensin system (RAS), which may if started early enough help slow the progression of kidney disease.

How is this medicine expected to work?

Scarring and kidney damage in patients with long-term kidney disorders, including Alport syndrome, is associated with production of high levels of a type of genetic material (RNA) called micro-RNA21.

This medicine is a short strand of synthetic RNA-like material that is able to attach to and neutralise micro-RNA21. By neutralising micro-RNA21, it is expected to reduce the development of scarring and kidney damage in patients with the disease, and so slow the loss of kidney function and delay the need for transplantation or dialysis.

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 this medicine in patients with Alport syndrome had been started.

At the time of submission, 5'-$A_SC_SA_ST_SC_SA_SG_ST_SC_ST_SG_SA_SU_SA_SA_SG_SC_ST_SA$-3' was not authorised anywhere in the EU for Alport syndrome. 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 12 February 2015 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:

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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	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Treatment of Alport syndrome
Bulgarian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	лечение на синдрома на Алпорт
Croatian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Liječenje Alportovog sindroma
Czech	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Léčba Alportova syndromu
Danish	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Behandling af Alports syndrom
Dutch	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Behandeling van het syndroom van Alport
Estonian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Alporti sündroomi ravi
Finnish	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Alportin oireyhtymän hoito
French	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Traitement du syndrome d'Alport
German	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Behandlung des Alport-Syndroms
Greek	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Θεραπεία του συνδρόμου Alport
Hungarian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Alport-szindróma kezelésére
Italian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Trattamento della sindrome di Alport
Latvian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Olporta sindroma ārstēšanai
Lithuanian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Alporto sindromo gydymui
Maltese	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Kura tas-sindrome ta' Alport
Polish	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Leczenia zespołu Alporta
Portuguese	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Tratamento da síndroma de Alport
Romanian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Tratamentul sindromului Alport
Slovak	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Liečbu Alportovho syndrómu
Slovenian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Zadravljenje Alportovega sindroma
Spanish	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Tratamiento del síndrome de Alport
Swedish	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Behandlingen av Alports syndrom
Norwegian	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Behandling av Alports syndrom
Icelandic	5'-$A_S C_S A_S T_S C_S A_S G_S T_S C_S T_S G_S A_S U_S A_S A_S G_S C_S T_S$A-3'	Meðferð við Alport-heilkenni

[1] At the time of designation