

7 May 2018  
EMA/125875/2018

## Public summary of opinion on orphan designation

### Recombinant modified ricin toxin A-chain subunit for the prevention of ricin poisoning

On 21 March 2018, orphan designation (EU/3/18/2001) was granted by the European Commission to Soligenix UK Ltd., United Kingdom, for recombinant modified ricin toxin A-chain subunit (also known as RiVax) for the prevention of ricin poisoning.

#### What is ricin poisoning?

Ricin is a highly toxic protein derived from the seeds of the castor oil plant. Ricin poisoning can be intentional (mainly by people attempting suicide) or can happen by mistake, for example in people researching or working with it. It is considered that ricin might also be used in acts of terrorism.

Initial symptoms of ricin poisoning depend on how the poison is given but may include fever, cough, nausea and vomiting (feeling and being sick), shock and difficulty breathing. Ricin poisoning is life-threatening due to multiple organ failure and usually leads to death within a few days.

#### What is the estimated number of people at risk of ricin poisoning?

At the time of designation, the number of people at risk of ricin poisoning was estimated to be less than 0.001 people in 10,000 in the European Union (EU). This was equivalent to a total of fewer than 50 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 of designation, no satisfactory methods were authorised in the EU for the prevention of ricin poisoning.

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\*Disclaimer: For the purpose of the designation, the number of patients at risk of developing 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 517,400,000 (Eurostat 2018).

## How is this medicine expected to work?

This medicine is a copy of part of the ricin toxin which has been inactivated by changing one of its amino acids (the building blocks of proteins). It works like a vaccine, by 'teaching' the immune system (the body's natural defences) how to defend itself against ricin poisoning. When the medicine is given to people at risk of ricin poisoning, the immune system recognises ricin as 'foreign' and makes antibodies against it. When the person is then exposed to ricin, these antibodies together with other components of the immune system will be able to destroy the toxin and help protect against poisoning.

## 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 people were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the prevention of ricin poisoning. Orphan designation of the medicine had been granted in the United States.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 2018 recommending the granting of this designation.

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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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant modified ricin toxin A-chain subunit	Prevention of ricin poisoning
Bulgarian	Рекомбинантна модифицирана субединица на А-веригата на рициновия токсин	Предотвратяване на отравяне с рициново масло
Croatian	Podjedinica A-lanca rekombinantno modificiranog ricinusovog toksina	Prevenција trovanja ricinusom
Czech	Modifikovaná rekombinantní podjednotka A řetězce ricinového toxinu	Prevence otravy ricinem
Danish	Rekombinant modificeret ricin toxin A-kæde subunit enhed	Forebyggelse af ricinforgiftning
Dutch	Recombinant gemodificeerd ricine toxine A-keten subunit	Preventie van ricinevergiftiging
Estonian	Rekombinantne ritsiini toksiini A-ahela modifitseeritud alaühik	Ritsiini mürgituse ennetamine
Finnish	Rekombinantti muokattu risiinitoksiinin A-ketjun alayksikkö	Rutiinimyrkytyksen estäminen
French	Sous-unité de la chaîne A de la toxine ricinique modifiée recombinante	Prévention de l'empoisonnement à la ricine
German	Rekombinantes modifiziertes Rizin Toxin A-Kette	Prävention von Rizin-Vergiftung
Greek	Ανασυνδυασμένη τροποποιημένη υπομονάδα Α-αλύσου της τοξίνης της ρικίνης	Πρόληψη της δηλητηρίασης με ρικίνη
Hungarian	Rekombináns módosított ricin toxin A-lánc alegysége	Ricin mérgezés megelőzése
Italian	Subunità della catena A della tossina della ricina modificata ricombinante	Prevenzione dell'avvelenamento da ricina
Latvian	Rekombinanta, modificēta ricīna toksīna A-ķēdes subvienība	Saīndēšanās ar ricīnu prevencija
Lithuanian	Rekombinantinis modifikuotas ricino toksino A-grandies subvienetas	Apsinuodijimo ricinu prevencija
Maltese	Subunità tal-katina A b'tossin ricina modifikat rikombinanti	Prevenzjoni ta' avvelenament bir-ricina
Polish	Rekombinowana podjednostka łańcucha A modyfikowanej toksyny rycyny	Zapobieganie zatruciu rycyną
Portuguese	Subunidade modificada da cadeia A da toxina ricina recombinante	Prevenção da intoxicação por ricina
Romanian	Subunitate a lantului A al toxinei ricinice modificată recombinantă	Prevenirea otrăvirii cu ricină
Slovak	Rekombinantná modifikovaná podjednotka A-reťazca ricínového toxinu	Prevenca otravy ricínom
Slovenian	Rekombinantna modificirana podenota A-verige toksina ricina	Preprečevanje zastrupitve z ricinom

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Spanish	Subunidad modificada recombinante de la toxina A de la ricina	Prevención de envenenamiento por la ricina
Swedish	Rekombinant modifierad A-kedjesubenhhet av ricintoxin	Förebyggande av ricinförgiftning
Norwegian	Rekombinant modifisert ricin toksin A-kjede subenhhet	Forebygging av ricin forgiftning
Icelandic	Raðbrigða breytt rísín eitur A keðju undireining	Forvarnir gegn ricin eitrun