27 May 2016
EMA/COMP/247747/2016
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Human/murine chimeric monoclonal antibody against endoglin for the treatment of soft tissue sarcoma



On 28 April 2016, orphan designation (EU/3/16/1648) was granted by the European Commission to Tracon Pharma Limited, United Kingdom, for human/murine chimeric monoclonal antibody against endoglin (also known as TRC105) for the treatment of soft tissue sarcoma.

### What is soft tissue sarcoma?

Soft tissue sarcoma is a cancer that affects the soft, supportive tissues of the body. It can occur in muscles, blood vessels, fat tissue or in other tissues that support, surround and protect organs. Patients with soft tissue sarcoma do not usually have symptoms in the early stages of the disease. First symptoms appear when the tumour grows large enough to cause swelling and pain.

Soft tissue sarcoma is a long-term debilitating and life-threatening disease, particularly when the cancer has spread to other parts of the body.

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

At the time of designation, soft tissue sarcoma affected approximately 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 113,000 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, the main treatment for early-stage soft tissue sarcoma was surgery. For large sarcomas, surgery was usually followed by radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer) to kill any cancer cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma including doxorubicin.

*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 513,700,000 (Eurostat 2016).

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The sponsor has provided sufficient information to show that medicine might be of significant benefit for patients with soft tissue sarcoma. This is because results from early studies show positive effects when used together with the cancer medicine pazopanib in patients previously treated with other cancer medicines. 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 a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein called endoglin. Endoglin is found in high amounts on growing blood vessels, including in sarcoma tumours where it is thought to contribute to the development of new blood vessels needed by the tumour to grow. By attaching to endoglin, this medicine is expected to prevent the tumour from being supplied by new blood vessels, eventually killing the tumour cells.

## 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 soft tissue sarcoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for soft tissue sarcoma 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 23 March 2016 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[1], Norwegian and Icelandic

| Language | Active ingredient | Indication |
|---|---|---|
| English | Human/murine chimeric monoclonal antibody against endoglin | Treatment of soft tissue sarcoma |
| Bulgarian | Човешко/мише химерично моноклонално антитяло срещу ендоглин | Лечение на сарком на меките тъкани |
| Croatian | Ljudsko/mišje kimerično monoklonalno antitijelo na endoglin | Liječenje sarkoma mekih tkiva |
| Czech | Lidská/myší chimérická monoklonální protilátka vůči endoglin | Léčba sarkomu měkkých tkání |
| Danish | Humant/murint kimerisk monoklonalt antistof mod endoglin | Behandling af bløddelssarkom |
| Dutch | Humaan/murien chimerisch monoklonaal antilichaam tegen endogline | Behandeling weke delen sarcoom |
| Estonian | Endogliini vastane inimese/hiire kimäärne monoklonaalne antikeha | Pehmete kudede sarkoomi ravi |
| Finnish | Ihmisen/hiiren kimeerinen monoklonaalinen endogliini-vasta-aine | Pehmytkudossarkooman hoito |
| French | Anticorps monoclonal chimérique humain/murin contre endogline | Traitement des sarcomes des tissus mous |
| German | Human/muriner chimärer monoklonaler Antikörper gegen Endoglin | Behandlung des Weichteilsarkoms |
| Greek | Χιμαιρικό μονοκλωνικό αντίσωμα ανθρώπου/μυός εναντίον της ενδογλίνης | Θεραπεία του σαρκώματος των μαλακών ιστών |
| Hungarian | Humán/egér kiméra eredetű endoglin ellenes monoklonális antitest | Lágy szöveti sarcoma kezelése |
| Italian | Anticorpo monoclonale chimerico topo-uomo anti endoglina | Trattamento dei sarcomi dei tessuti molli |
| Latvian | Cilvēka/peļu himēriska monoklonāla antiviela pret endoglīnu | Mīksto audu sarkomas ārstēšana |
| Lithuanian | Chimerinis žmogaus/pelės monokloninis antikūnas prieš endogliną | Minkštųjų audinių sarkomos gydymas |
| Maltese | Antikorp monoklonali kimeriku uman/tal-ġrieden kontra l-endoglin | Kura tas-sarkoma tat-tessuti rotob |
| Polish | Ludzkie/mysie monoklonalne chimeryczne przeciwciało przeciwko endoglinie | Leczenie mięsaków tkanek miękkich |
| Portuguese | Anticorpo monoclonal quimérico murino/humano contra endoglina | Tratamento do sarcoma dos tecidos moles |
| Romanian | Anticorp monoclonal chimeric uman/murin îndreptat împotriva endoglinei | Tratamentul sarcomului ţesuturilor moi |
| Slovak | Ľudská/myšia chimérická monoklonálna protilátka proti endoglínu | Liečba sarkómu mäkkých tkanív |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Slovenian | Humano/mišje monoklonsko himerno protitelo proti endoglinu | Zdravljenje sarkoma mehkih tkiv |
| Spanish | Anticuerpo monoclonal quimérico humano/murino contra endoglina | Tratamiento del sarcoma de tejidos blandos |
| Swedish | Human/murin chimerisk monoklonal antikropp mot endoglin | Behandling av mjukdelssarkom |
| Norwegian | Humant/murint kimerisk monoklonalt antistoff mot endoglin | Behandling av bløtvevssarkom |
| Icelandic | Einstofna manna/músa blendingsmótefni úr gegn endóglíni | Meðferð við mjúkvefjasarkmeini |