

6 May 2015  
EMA/COMP/123186/2015  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Recombinant human club cell 10 KDa protein for the prevention of bronchopulmonary dysplasia

On 19 March 2015, orphan designation (EU/3/15/1456) was granted by the European Commission to RLM Consulting, Belgium, for recombinant human club cell 10 KDa protein for the prevention of bronchopulmonary dysplasia.

#### What is bronchopulmonary dysplasia?

Bronchopulmonary dysplasia is a lung disease affecting premature babies who have been on prolonged mechanical ventilation (using a machine that supplies oxygen to help with breathing). In premature babies breathing difficulties can occur because the lungs are immature and do not have enough surfactant, which is a substance lining the lungs and airways that allows them to expand properly. Mechanical ventilation may therefore be needed to force oxygen into the lungs. However, the constant high pressure of the oxygen supplied through mechanical ventilation causes inflammation and injury to the lungs and blocks the normal development of the alveoli (air sacs in the lungs). This leads to long-term breathing problems and weight loss.

Bronchopulmonary dysplasia is a long-term and life-threatening condition because the damaged and underdeveloped lungs may not work properly.

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

At the time of designation, the number of patients at risk of bronchopulmonary dysplasia was estimated to be between 1 and 3 people in 10,000 in the European Union (EU). This was equivalent to a total of between 51,000 and 154,000 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).

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\*Disclaimer: For the purpose of the designation, the number of patients at risk of 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).

## **What methods of prevention are available?**

At the time of designation, no satisfactory methods of prevention were authorised in the EU for bronchopulmonary dysplasia.

## **How is this medicine expected to work?**

This medicine is made of a protein called 'club cell 10 KDa protein' (CC10) that is found in high amounts in the lining of the lungs of healthy individuals but in very small amounts in premature babies. CC10 works by reducing inflammation. By giving the medicine directly into the lungs of premature babies, it is expected that the medicine will reduce inflammation and damage to the lungs.

## **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 premature babies were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for prevention of bronchopulmonary dysplasia. Orphan designation of the medicine had been granted in the United States for the prevention of neonatal bronchopulmonary dysplasia in premature neonates with respiratory distress syndrome (breathing difficulties).

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.

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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:

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

| Language   | Active ingredient                                                                 | Indication                                    |
|------------|-----------------------------------------------------------------------------------|-----------------------------------------------|
| English    | Recombinant human club cell 10 KDa protein                                        | Prevention of bronchopulmonary dysplasia      |
| Bulgarian  | Рекомбинантен човешки протеин 10 KDa от бронхиоларни екзокринни клетки            | Предотвратяване на бронхопулмонална дисплазия |
| Croatian   | Rekombinantni ljudski protein od 10 kDa iz egzokrinih stanica bronhiola           | Prevenција bronhopulmonalne displazije        |
| Czech      | Rekombinantní lidský protein bronchiolárních exokrinních buněk o hmotnosti 10 KDa | Prevence bronchopulmonální dysplasie          |
| Danish     | Rekombinant human club-celle 10 kDa protein                                       | Forebyggelse af bronkopulmonal dysplasi       |
| Dutch      | Recombinant humaan club celeiwit van 10 kDa                                       | Preventie van bronchopulmonale dysplasie      |
| Estonian   | Inimese club-rakue rekombinantne 10 KDa valk                                      | Bronhopulmonaarse düsplaasia ennetamine       |
| Finnish    | Ihmisen club-solun 10 KDa:n rekombinanttiproteiini                                | Bronkopulmonaarisien dysplasian ehkäisy       |
| French     | Protéine humaine recombinante de cellule club de 10 KDa                           | Prévention de la dysplasie broncho-pulmonaire |
| German     | Rekombinantes menschliches 10-kDa Clubzell-Protein                                | Prävention von bronchopulmonaler Dysplasie    |
| Greek      | Ανασυνδυασμένη ανθρώπινη πρωτεΐνη κυττάρου club 10 kDa                            | Πρόληψη της βρογχοπνευμονικής δυσπλασίας      |
| Hungarian  | 10 kDa molekulatömegű rekombináns humán club-sejt-fehérje                         | Bronchopulmonalis dysplasia megelőzése        |
| Italian    | Proteina 10 KDa della cellula club umana ricombinante                             | Prevenzione della displasia broncopulmonare   |
| Latvian    | Rekombinanta cilvēka bronhiolu sekretšunu 10 KDa olbaltumviela                    | Bronhopulmonālās displāzijas novēršana        |
| Lithuanian | Rekombinantinis žmogaus bronchiolių egzokrininių ląstelių 10 KDa baltymas         | Bronchopulmoninės displazijos prevencija      |
| Maltese    | Proteina 10 KDa ta' ċelloli club umani rikombinanti                               | Prevenzjoni ta' displasja bronkopulmonari     |
| Polish     | Rekombinowane ludzkie białko z komórek oskrzelikowych o ciężarze 10 kD            | Zapobieganie dysplazji oskrzelowo-płucnej     |
| Portuguese | Proteína humana recombinante de célula club de 10 Kda                             | Prevenção de displasia broncopulmonar         |
| Romanian   | Proteină umană recombinantă de 10 kDa din celule bronhiolare exocrine             | Prevenirea displaziei bronhopulmonare         |
| Slovak     | Rekombinantný ľudský proteín clubových buniek o veľkosti 10 kDa                   | Prevenčia bronchopulmonálnej dysplázie        |

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

| Language  | Active ingredient                                                                | Indication                                |
|-----------|----------------------------------------------------------------------------------|-------------------------------------------|
| Slovenian | Rekombinantni humani protein alveolarnih celic tipa II, z molekulsko maso 10 KDa | Preprečevanje bronhopulmonalne displazije |
| Spanish   | Proteína recombinante de célula exocrina bronquiolar humana de 10 KDa            | Prevención de la displasia broncopulmonar |
| Swedish   | Rekombinant mänsklig claracell 10 KDa protein                                    | Prevention av bronkopulmonell dysplasi    |
| Norwegian | Rekombinant humant Clara-celle 10 KDa-protein                                    | Forebygging av bronkopulmonal dysplasi    |
| Icelandic | Raðbrigða manna klúbb fruma 10 KDa prótein                                       | Til að fyrirbyggja berkjulungna misþroska |