Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decision[i]

Actives substances(s): human recombinant interleukin-2 / Aldesleukin

Latest Decision number(s): 1) P/0097/2015

Corresponding PIP number(s): 1) EMEA-001556-PIP01-13

If the PIP has been submitted as part of a marketing authorisation application in order to comply with the requirements of Article 7 of the Paediatric Regulation (as a condition of the validation of the respective application) and a marketing authorisation was granted based on this application, then there is a legal obligation to complete that PIP. The same applies if there has been a successful post-authorisation application, where the PIP was included in order to comply with the requirements of Article 8 of the Paediatric Regulation.

Please confirm if any of the above applies:

Yes ☐ No ☒

If yes, it means that based on the Marketing Authorisation obtained at the end of that initial procedure or the successful post-authorisation application, as applicable, you are obliged to complete that PIP. That obligation cannot be cancelled by a unilateral decision, including by withdrawing the MA. Such PIP must be completed, unless it is modified in agreement with the PDCO by removing all outstanding PIP measures or granting a full product-specific waiver instead (upon relevant circumstances in accordance with the Paediatric Regulation). Non-completion of a binding PIP establishes noncompliance with the requirements of the Paediatric Regulation, which the European Medicines Agency has an obligation to report to the European Commission.

Please note that development of the medicinal product above in the following **condition(s)/indication(s)**:

☒ has been discontinued

for the following reason(s): (tick all that apply)

☒ (possible) lack of efficacy in adults

☒ (possible) lack of efficacy in children

☐ (possible) unsatisfactory safety profile in adults

☐ (possible) unsatisfactory safety profile in children

☐ commercial reasons (please specify:)

☐ manufacturing / quality problems

☐ other regulatory action (please specify:)

☐ other reason (please specify:)

Please add a brief description (max 2000 characters) of the reason(s) for the discontinuation:

We have conducted extensive research into the safety of IL-2 in mice (Churlaud et al., Clinical Immunology, 2014; PMID: 24576619). We followed juvenile mice from week 4 onwards that were treated with IL-2 via an AAV injection, which resulted in permanent exposure to IL-2. We did not observe any adverse effects in these settings.

We have also conducted two clinical studies in children:

- Df-IL2child was a multicentre, double-blind, placebo-controlled, dose-finding study of IL-2 in children with recently diagnosed type 1 diabetes. Twenty-four patients were randomly assigned in a 1:1:1:1 ratio to receive either a placebo or IL-2 at doses of 0.125, 0.250, or 0.500 million international units (MIU)/m², administered daily for five days and then fortnightly for one year. There were no serious adverse events. Non-serious adverse events (NSAEs) were transient and mild to moderate. The results have been published (Rosenzwajg et al., Diabetologia, 2020; PMID: 32607749).

- Diabil-2 was a multicentre, double-blind, randomised, placebo-controlled, phase 2b dose-finding trial. Participants aged 6–35 years were randomised 1:1:1 to receive IL-2 low-dose according to either Regimen A or B or a placebo. They were also randomised according to their pubertal age. Treatment began with daily subcutaneous injections for five days followed by fortnightly injections for Regimen A or weekly injections for Regimen B for 12 months. Adults received one million international units (MIU) per injection, while children with a Tanner score of ≤4 received 0.5 MIU/m². A total of 141 patients (75 adults and 66 children) were enrolled. The DIABIL-2 study did not show any significant differences between ILT-101 and placebo in terms of the primary endpoint (a change from baseline in stimulated C-peptide area under the curve (AUC) during a mixed meal tolerance test at 12 months) or secondary efficacy endpoints (change in HbA1c, insulin requirements and fasting metabolic parameters such as glucose and C-peptide) in both adults and children. IL-2 low-dose was well tolerated across age groups and regimens, with no significant safety concerns. A manuscript is about to be sent for publication.

This led to the Applicant's decision to no longer pursue further adult and paediatric development of ILT-101 for the treatment of type 1 diabetes mellitus.

Name and signature of the PIP contact point:

Date: 19/03/2026

Contact for inquiries from interested parties:

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[i] This form will be published to the corresponding decision available on the website of the European Medicines Agency.