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National Health Care Institute
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Our reference
2025008932

Date 17 April 2025
Re: Advice about early termination of the conditional inclusion procedure of ataluren (Translarna®)

Dear Ms Agema,

Ataluren (Translarna®) is conditionally included in the basic healthcare package from 1 November 2021 until 1 October 2025. Previously, we advised you to terminate the conditional inclusion (CI) of ataluren early if the European Commission (EC) adopts the negative advice issued by the Committee for Medicinal Products for Human Use (CHMP).[1] This has been the case since 28 March 2025.[2] In this letter we therefore advise you to stop the CI procedure immediately.

Registration history and course of the CI procedure of ataluren (Translarna®)
On 31 July 2014, the European Medicines Agency (EMA) registered ataluren for the treatment of Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene (nmDMD) in ambulatory patients aged two years and older. Ataluren was qualified as a conditional. This means that the medicinal product was admitted to the market subject to additional studies.

On 7 October 2021, the National Health Care Institute advised your predecessor to admit ataluren to the basic healthcare package on a conditional basis from 1 November 2021 until 1 February 2024.[3] The National Health Care Institute indicated to start the final assessment with a ruling on inclusion in the package after the EC decided that ataluren remains on the market. As a result, we advised you twice to extend the CI procedure.[4][5], In our last advice, dated 6 August 2024, we recommended an end date of 1 October 2025.4

Following a lengthy process in the EMA, during which the CHMP repeatedly issued negative advice on the renewal of the marketing authorisation of ataluren, the EC decided on 28 March 2025 to adopt the CHMP's negative advice and to not renew

[1] Advice - Early termination of conditional inclusion of medicinal products rhPTH 1-84 (Natpar®) and ataluren (Translarna®) | Advice | National Health Care Institute
[2] dec_160780_nl.pdf
[3] Additional advice on conditional inclusion of ataluren (Translarna®) | Advice | National Health Care Institute
[4] Advice - extend conditional inclusion procedure ataluren (Translarna®) | Advice | National Health Care Institute
[5] Advice - extend conditional inclusion procedure ataluren (Translarna®) | Advice | National Health Care Institute

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the marketing authorisation. The CHMP concluded that the effectiveness of ataluren has not been established for the authorised indication, nor for any subpopulation of this indication. A favourable benefit-risk balance was not confirmed. This means that PTC Therapeutics – the marketing authorisation holder for ataluren – will no longer be authorised to supply ataluren in the European Union (EU) and the European Economic Area (EEA). However, in its decision, the EC has stated that countries can individually make an appeal to Articles 117(3) and 5(1) of the EU Directive 2001/83 for the continued use of ataluren. However, this is outside the scope of the CI. With ataluren no longer registered in the EU and EEA, participants in the CI can no longer obtain ataluren. As the effectiveness of ataluren has not been established by the EMA, we recommend that you terminate the CI procedure for ataluren immediately.

Conclusion of the CI procedure
Shortly after it became known that the conditional market authorisation was not extended, the marketing authorisation holder informed the National Health Care Institute and stakeholders involved in the CI procedure for ataluren about this. They met on 7 April to discuss the conclusion of the CI procedure. It is regrettable for patients and their loved ones that ataluren has been taken off the market because of the seriousness of the condition and the large, unmet medical need. The treating physicians indicated that they would immediately inform the patients who are still being treated with ataluren. Spierziekten Nederland, the Dutch patient association that advocates on behalf of patients with Duchenne muscular dystrophy, indicated that an information evening for patients and their families would be organised within the next few weeks. The professional association and the patient association are joining forces in this effort. The National Health Care Institute is confident that the CI procedure will be completed appropriately and that patients and their loved ones will be well informed and supported when treatment with ataluren is stopped.

In conclusion
Because the effectiveness of ataluren, according to the EMA, has not been established for the authorised indication, nor for any subpopulation of this indication, and because, as a result of this, the registration of the product has not been renewed, ataluren can no longer be reimbursed through CI. We advise you to stop the CI procedure for ataluren (Translarna®) immediately. The National Health Care Institute will not be conducting a package assessment of ataluren.

Please do not hesitate to contact me should you have any further questions.

Yours sincerely,

M.J. Janssen
Chairperson of the Executive Board