Zorginstituut Nederland

> Return address PO Box 320, 1110 AH Diemen

To the Minister of Health, Welfare and Sport
P.O. Box 20350
2500 EJ THE HAGUE

National Health Care Institute Research, Development and Medicinal Products
Medicinal Products Team

Willem Dudokhof 1
1112 ZA Diemen
PO Box 320
1110 AH Diemen
www.zorginstituutnederland.nl
info@zinl.nl

T +31 (0)20 797 85 55

Contact
A. van der Waal
vragen@zinl.nl

Our reference
2026005486

2026005486

Date 11 March 2026
Re: Progress report on the conditional inclusion of orphan drugs, conditionals and exceptionals

Dear Ms Hermans,

In this letter, we explain the progress of the conditional inclusion (CI) of orphan drugs, conditionals and exceptionals for the reporting year 2025.

With the CI procedure for orphan drugs, conditionals and exceptionals, the National Health Care Institute is committed to proactive package management to promote appropriate care. Through this procedure, patients with a serious condition and unmet medical need may be eligible for reimbursement of promising medicinal products from the basic health insurance package, even though the products do not yet meet the established medical science and medical practice (SWP). After sufficient evidence has been collected for the verification on SWP, the medicinal product either remains in the basic health insurance package after a positive SWP assessment and successful price negotiations, or it is removed from the basic health insurance package after a negative SWP assessment or unsuccessful price negotiations.

In 2025, two medicinal products were conditionally included in the basic health insurance package: ataluren (Translarna) and teduglutide (Revestive®).

Ataluren (Translarna®)
In the progress report for the year 2024, we advised you to terminate the CI process for ataluren (Translarna®) early if the European Commission (EC) adopted the negative advice from the Committee for Medicinal Products for Human Use (CHMP) on renewing the market authorisation for ataluren.[1]Following a lengthy process in the European Medicines Agency (EMA), during which the CHMP repeatedly issued negative advice on the renewal of the market authorisation of ataluren, the EC decided on 28 March 2025 to adopt the CHMP's negative advice and not renew the market authorisation.[2] Studies showed that there is no difference in effectiveness between people who received ataluren and people who received placebo. Since ataluren was no longer registered in the EU from that moment, participants in the CI could no longer obtain ataluren. On 17

[1] Voortgangsrapportage+voorwaardelijke+toelating+weesgeneesmiddelen,+conditionals+en+exceptionals+(voortgangsrapportage+verslagjaar+2024) (1).pdf

[2] dec_160780_nl.pdf

April 2025, we advised you to stop the CI process for ataluren immediately.[3] As of 1 June 2025, ataluren was removed from List 1B of the Health Insurance Regulations.[4] Thus, the conditional inclusion to the basic health insurance package of ataluren for the treatment of *'an ambulatory insured person aged two years and older with Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene'* has ended. All patients have since finished treatment with ataluren.

Teduglutide (Revestive®)
Teduglutide was conditionally included from 1 April 2025 to 1 April 2030 in the basic health insurance package for the treatment of certain patients with short bowel syndrome. In December 2025, the National Health Care Institute attended the sounding board meeting for teduglutide. During this meeting, the progress and relevant interim findings of the study were discussed with all stakeholders. In addition, we have received a written progress report from the stakeholders. On this basis, the National Health Care Institute has prepared a progress report, which is attached as an Annex to this letter. The National Health Care Institute does not see any reason for the adjustment or early discontinuation of the CI process for teduglutide.

Preview 2026
The National Health Care Institute hopes to start at least two new CI processes this year. Last month, we advised you to conditionally include atidarsagene autotemcel (Libmeldy®) for a specific group of patients with the rare and hereditary metabolic disease metachromatic leukodystrophy (MLD) in the basic health insurance package. The financial arrangement for atidarsagene autotemcel has already been successfully concluded. If you adopt the advice on atidarsagene autotemcel, this CI process will be ready to start soon. Risdiplam (Evrysdi®) was identified as a potential candidate for CI in 2025. If the covenant phase of risdiplam is successfully completed and you agree with the marketing authorisation holder on a reduced price during CI, it is expected that this product will also be able to start a CI process in 2026.

Yours sincerely,

M.J. Janssen
Chairperson of the Executive Board

National Health Care Institute
Research, Development and Medicinal Products
Medicinal Products Team

Date
11 March 2026

Our reference
2026005486

[3] Advies+over+vroegtijdige+beëindiging+van+voorwaardelijke+toelating+van+ataluren+(Translarna®).pdf

[4] Parliamentary paper 2025, no. 19703 | Overheid.nl > Official publications

Annex: Progress report teduglutide (Revestive®) for the treatment of certain patients with short bowel syndrome

National Health Care Institute
Research, Development and Medicinal Products
Medicinal Products Team

Date
11 March 2026

Our reference
2026005486

Teduglutide (Revestive®)

Teduglutide was conditionally included from 1 April 2025 to 1 April 2030 in the basic health insurance package for the treatment of a specific patient group aged six months and older with short bowel syndrome. This is a patient:

- aged 18 years and older with
  - Inflammatory Bowel Disease (IBD) as a cause of intestinal resection who is dependent, at least 3 times a week, on total parenteral nutrition (TPN) for at least 12 months, or,
  - a parenteral nutrition volume greater than 14 litres/week for at least 12 months;
- aged 6 months to 18 years who is dependent on parenteral support for at least 30% of their caloric and/or fluid and electrolyte need with no or a minimal increase in enteral nutrition in the past 3 months, where natural adaptation is excluded as far as possible (according to the starting criteria formulated by the physicians' association).

Short bowel syndrome is a rare condition in which the small intestine is usually mostly absent or not functioning properly (intestinal failure). In particular, reduced nutrient uptake can cause problems such as diarrhoea, weight loss and fatigue. People with short bowel syndrome often receive parenteral nutrition. Long-term use of this may be associated with rare but potentially life-threatening complications, such as liver disease. Furthermore, the use of chronic parenteral nutrition is a heavy burden on the patient, with a negative impact on the quality of life. Teduglutide promotes recovery and normal growth of the intestine, allowing the required amount of TPN to be reduced.

The number of Dutch patients treated with teduglutide within the conditional inclusion period was estimated to be no more than 90.

Study progress

Study design
This is a prospective, observational registry study with intra-patient comparisons. Dutch patient data is included in the Revestive Monitor.

Number of patients included
Due to delivery problems of teduglutide, actual inclusion started on 29 September 2025. By 31 December 2025, a total of 12 patients had been included: 3 adults and 9 children. In addition, several patients are scheduled for inclusion to start teduglutide in 2026.

Assessment by the indication committee
In 2025, 4 adults were assessed by the indication committee. One patient did not meet the start criteria. This was a patient having had a small intestine transplant with a high-output ileostomy, so anatomically not short bowel syndrome.

In 2025, 13 children were assessed and approved by the indication committee. One child did not start, but was admitted at the parents' request to reduce the TPN without teduglutide.

National Health Care Institute
Research, Development and Medicinal Products
Medicinal Products Team

Date
11 March 2026

Our reference
2026005486

Participating hospitals
Based on the current start criteria, 25 of the 74 patients with short bowel syndrome who are dependent on parenteral support at the Amsterdam UMC are eligible to participate in the CI for teduglutide. Not all 25 patients have been approached yet. One patient refused treatment and one person was excluded from participation by the treatment team.

The UMC Groningen is currently carrying out an inventory of the relevant patient population. Only a limited number of patients are expected to meet the start criteria.

The Radboud University Medical Center is expected to refer 3 children to the Amsterdam UMC for treatment with teduglutide.

Envisaged problems with the conditional reimbursement – and thus the data collection – for teduglutide have been solved
During the sounding board meeting, there was uncertainty about the reimbursement for patients <18 years of age who would come of age during the treatment. This has been clarified by amending the physician statement. Patients who become adults during treatment meet the paediatric criteria at the start of teduglutide treatment (6 months up to 17 years, ≥30% dependence on TPN), but formally no longer comply once they reach the age of 18. At the same time, these young adults, especially when treatment has been effective and the TPN is now being reduced, do not meet the adult criteria. It has been agreed to continue treatment after transition. At baseline, both the Quality of Life questionnaire for children and adults is administered in children who reach the age of 18 during the CI period. In addition, the physician's statement has been amended to require patients to comply with the List 2 conditions *at the start of treatment* as from 1 February. This prevents patients from no longer meeting the initial criteria of the physician's statement in case of effective treatment and from being unjustifiably unable to obtain reimbursement of teduglutide.

Developments in international literature

No new literature has been published showing that teduglutide shows a clinically relevant improvement of the quality of life in other subgroups of adult patients with short bowel syndrome and intestinal failure. There is also currently insufficient direct evidence available to demonstrate the cost-effectiveness of GLP-2 convincingly.[5]

No new literature on the (cost) effectiveness of teduglutide in children with short bowel syndrome and intestinal failure has been published since the start of the CI.

Vision of the physicians' association

The physicians' association believes that the inclusion criteria for adults are too strict and unfair and wants more patients to have access to teduglutide. For example, for people with a lower body weight, it is impossible to have a parenteral feeding volume greater than 14 litres/week. During the sounding board meeting, the physicians' association therefore requested that the start and stop

[5] Cost-effectiveness of teduglutide in adult patients with short bowel syndrome - a European socioeconomic perspective - PubMed

National Health Care Institute
Research, Development and Medicinal Products
Medicinal Products Team

Date
11 March 2026

Our reference
2026005486

criteria of teduglutide be adjusted for adults. The National Health Care Institute indicated that it was important for parties to gain experience with the current start and stop criteria at least during the coming year to evaluate the effects of teduglutide on the currently defined subgroup. If the experiences are positive and there is evidence from peer-reviewed scientific publications showing that there are also other subgroups in which teduglutide shows a clinically relevant improvement of the quality of life, this may give rise to a reassessment of the start and stop criteria after a year and to the submission of this proposal to the Scientific Advisory Board's Medical Products Committee (WAR CG).

The physicians involved do not agree with the reasoning of the National Health Care Institute. Therefore, they will provide further evidence in a letter as to why, in their opinion, the start criteria for CI should be expanded. This will clarify the number of patients this expansion would affect.

Unmet treatment need

There is currently no SWP-compliant alternative treatment available for children or adults with short bowel syndrome and intestinal failure. Similar products such as glepaglutide and apraglutide and a biosimilar of teduglutide are expected to be registered for adults in 2026. For children, this will take longer. At present, there is still an unmet medical need for both adults and children.

Conclusion on progress

After a delayed start due to delivery issues, the first patients have been included since September 2025. The National Health Care Institute does not see any reason for the adjustment or early discontinuation of the CI process for teduglutide at this time.