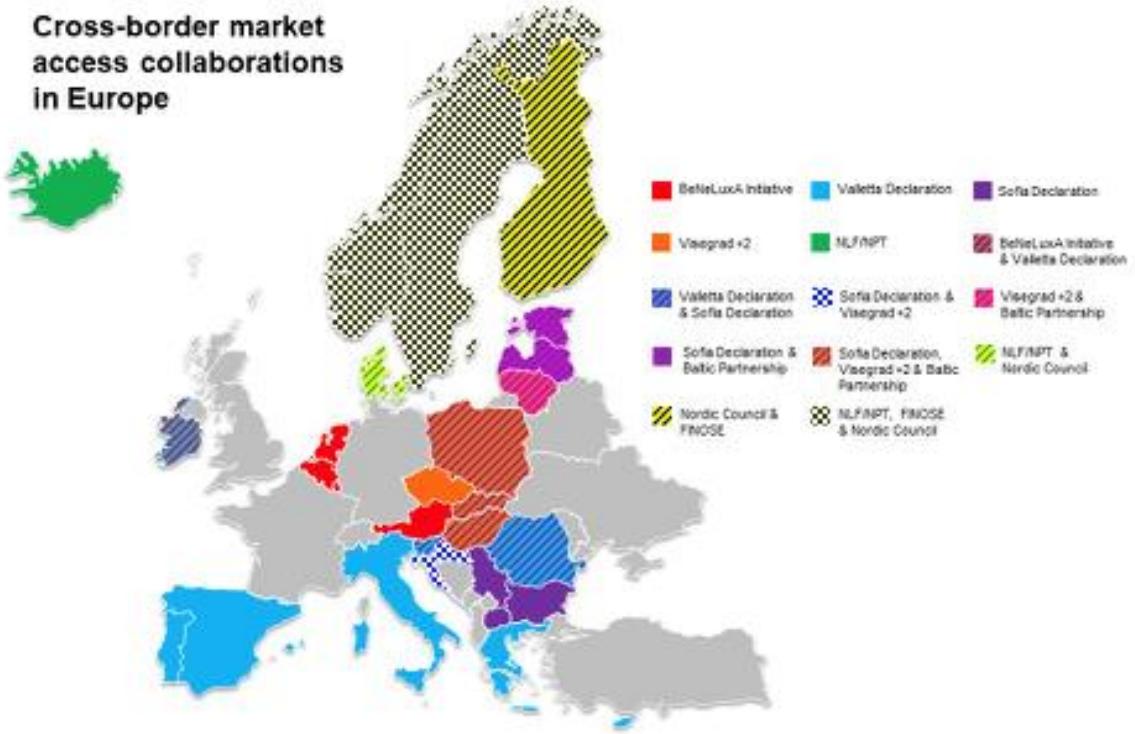
Cross Country Collaboration – the BeNeLuxA Initiative

Beneluxa
Initiative on Pharmaceutical Policy

Cross-border market access collaborations in Europe



- April 2015 Netherlands and Belgium announced the initiative.
- Sept 2015 Luxembourg joined.
- 2016 Austria joined - initiative became known as BeNeLuxA
- 2018 Ireland joined.

Beneluxa

Four Core Domains

- **DTF Horizon scanning**
- **DTF HTA**
- **DTF P&R**
- DTF Information Sharing

DTF HS

- IHSI
- DTF HS
  - Will directly inform which HTAs are of interest
  - Inform where short statements are needed or brief reports
  - Provide strategic signposts.

Pharmaceutical Developments on Haemophilia

This report, written by the Taskforce Horizon Scanning of the Beneluxa Initiative, describes the current situation of Haemophilia and provides an overview of new pharmaceutical developments regarding this disease. The current treatment options are listed, as well as the pharmaceutical costs in some of the Beneluxa countries.

Pharmaceutical Developments on Haemophilia

This report, written by the Taskforce Horizon Scanning of the Beneluxa Initiative, describes the current situation of Haemophilia and provides an overview of new pharmaceutical developments regarding this disease. The current treatment options are listed, as well as the pharmaceutical costs in some of the Beneluxa countries.

Pharmaceutical Developments on Alzheimer's Disease

This report, by the Taskforce Horizon Scanning of the Beneluxa Initiative, describes the current situation of Alzheimer's disease and provides an overview of new pharmaceutical developments regarding this disease.

The current treatment options are listed, as well as the available guidelines and an estimation of the current pharmaceutical costs for this disease in the Beneluxa countries.

DTF HTA

- Re-use of Health Technology Assessment (HTA) reports
- Joint writing of an HTA report

To date

- Short Statements.
- BeNeLuxA template for dossier submission.
- Aligning of processes

+ Background documents
+ About Beneluxa - press releases
+ About Beneluxa - statements
+ Templates

About Beneluxa - statements

19 May 2020: Joint HTA assessment of Zolgensma

On May 19 the European Commission granted conditional approval for Zolgensma (onasemnogene abeparvovec) for the treatment of patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1; or for patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to three copies of the SMN2 gene.

The Beneluxa Initiative welcomes a dialogue with the company AveXis/Novartis on access and affordability of the product in each country. Belgium, Ireland and the Netherlands aim to undertake a joint HTA assessment of Zolgensma as part of a reimbursement application by the manufacturer, with Austria acting as the reviewing expert in the procedure. The company AveXis/Novartis has stated its willingness to collaborate on this joint assessment and dialogue on reimbursement. As a consequence, HTA agencies in the three countries will align on the timing and content of the local HTA procedures. Joint price negotiations within the Beneluxa Initiative always start with a joint HTA procedure. Based on the outcome of the HTA assessment,countries will determine if the joint assessment will be followed by a joint price negotiation.

25 February 2020: Beneluxa Position Statement on CAR-T

Because of considerable upfront costs, CAR-T(°) treatments are in the process of being reviewed by a number of countries to determine whether they represent value for money. Many countries have determined that the early evidence indicates a benefit, but there is uncertainty due to the single-arm nature of the trials and the lack of data in the longer term. The impact of hematopoietic stem cell transplantation in patients treated with CAR-T further adds to the uncertainty as to whether the CAR-T is responsible for the possible prolongation of survival or whether the stem cell transplant primarily contributes to this.

Clinicians have indicated that a proportion of patients in paediatric acute lymphoblastic leukaemia who receive CAR-T are likely to continue to receive stem cell transplants. In determining comparative and cost effectiveness, the influence of this choice of treatment pathway should be carefully considered.

(°) Chimeric antigen receptor-engineered T-lymphocytes

+ Background documents
+ About Beneluxa

followed by a joint price negotiation.

25 February 2020: Beneluxa Position Statement on CAR-T

Because of considerable upfront costs, CAR-T(°) treatments are in the process of being reviewed by a number of countries to determine whether they represent value for money. Many countries have determined that the early evidence indicates a benefit, but there is uncertainty due to the single-arm nature of the trials and the lack of data in the longer term. The impact of hematopoietic stem cell transplantation in patients treated with CAR-T further adds to the uncertainty as to whether the CAR-T is responsible for the possible prolongation of survival or whether the stem cell transplant primarily contributes to this.

Clinicians have indicated that a proportion of patients in paediatric acute lymphoblastic leukaemia who receive CAR-T are likely to continue to receive stem cell transplants. In determining comparative and cost effectiveness, the influence of this choice of treatment pathway should be carefully considered.

(°) Chimeric antigen receptor-engineered T-lymphocytes

30 Jan 2020: No "lottery for life" - Statement by Beneluxa Health Ministers addressing the global managed access program designed by Novartis and Avexis

The Health Ministers of Belgium, the Netherlands, Luxembourg, Austria and Ireland have strong reservations in relation to the plans of Novartis and AveXis to let chance decide which seriously ill children will receive medication before it reaches the European market.

The approach set by the global managed access program is unprecedented and differs significantly from established early access schemes.

The Ministers – united in the Beneluxa-initiative – urge pharmaceutical companies to use objective medical criteria when they allow early access to innovative medicines.

The pharmaceutical company Novartis has recently applied for Market Authorization at the European Medicines Authority (EMA) for its product Zolgensma ®, for the treatment of Spinal Muscular Atrophy. By announcing this approach, Novartis has raised hope throughout patient communities but has remained silent on the exact plan and the legal challenges of such a system.

In their joint statement, the Ministers express strong concern at the organisation of a lottery-like approach for patients who are eagerly awaiting potential treatment: "*The high level of uncertainty and the non-transparent approach is unacceptable. It proves no sincere commitment to patients and only increases the distress of the families concerned. They are given false hope. If one equals the fate of a patient to a lottery ballot, human dignity and moral values get out of sight. Lotteries are by their nature a form of gambling and this is absolutely the wrong model to bring to healthcare.*"

(°) Chimeric antigen receptor-engineered T-lymphocytes

30 Jan 2020: No "lottery for life" - Statement by Beneluxa Health Ministers addressing the global ... program designed by Novartis and Avexis

The Health Ministers of Belgium, the Netherlands, ... Novartis and AveXis to let ...

28 May 2021 - Beneluxa Statement on high cost SMA treatments

Pharmacological treatments of Spinal Muscular Atrophy (SMA) have expanded across the full spectrum of the disease. Onasemnogene abeparvovec targets disease in patients with SMA who are presymptomatic or who are type 1 symptomatic, and less than 6 months of age. Nusinersen has a broad licence encompassing type 1 to type 3 SMA and includes adulthood. Risdiplam, which received CHMP positive opinion in February 2021, is the first oral treatment aimed at treating patients from 2 months of age into adulthood (Type 1 to 3).

It is possible that patients would receive at least 2 different of these treatments over their lifetime. Further, there are additional treatments in the pipeline. Currently the evidence base at point of regulatory approval mainly considers these treatments as stand alone. However, sequential use of at least two of these therapies, is not outside the scope of the licences, and has been observed in the clinical setting. Moreover, evidence of the long-term efficacy and safety of these treatments is lacking.

Given the considerable uncertainty associated with the long term effect of these treatments, health technology assessors and payers should incorporate the potential sequential costs of these high cost treatments. The (financial) risk associated with the lack of long term data and potential use of sequential treatments should not be carried by payers alone.

...aged access

...n to the plans of ...pean market.

...arly access

...they allow

...(EMA) for its ...pe

...are eagerly

...ceptor-engineered T-lymphocytes

+ Background documents
+ About Beneluxa
+ About B
+ Template

About Beneluxa - statements

29 June 2021 - Short Statement on Alzheimer's HTA

The Beneluxa group is following with attention the therapies relative to Alzheimer's disease coming to the market. Recently the FDA in the US approved an anti-amyloid antibody amid some controversy; a decision from EMA is not yet available.
Beneluxa wishes to emphasize that clinical endpoints such as changes in cognitive scores or dementia scales are considered as the endpoints relevant for HTA agencies in Alzheimer's clinical trials. Biomarker data are not considered sufficient for demonstration of benefit for HTA agencies.

04 June 2021 - Joint statement of the Beneluxa Initiative and the Nordic Pharmaceutical Forum

In recent years, efforts to enhance international payer collaboration and the willingness to exchange expertise and knowledge have increased substantially. The need for international exchange intensifies due to the challenges that authorities, including payers face concerning pricing, reimbursement and procurement of medicines and therapies. In order to ensure access to new medicines for patients these issues are essential to solve in an effective and rational way. The efforts have materialised in the form of international platforms such as the Nordic Pharmaceutical Forum (NLF); constituting of Norway, Sweden, Finland, Denmark and Iceland, and the Beneluxa Initiative; constituting of Belgium, the Netherlands, Austria, Ireland and Luxembourg. Concrete outcomes of these platforms include the organisation of the international horizon scanning initiative (IHSI) as well as an increased interaction between these platforms. A number of strategic meetings have taken place where other international payers also joined these discussions. Recently, the European Commission has launched a new Pharmaceutical Strategy for Europe highlighting similar issues with regards to ensuring access to affordable medicines. The Strategy specifically refers to the strengthening of existing and potential new cross-country collaborations.

arvovec) for the treatment of
diagnosis of SMA Type 1; or
ene.

of the product in each
rt of a reimbursement
AveXis/Novartis has stated
TA agencies in the three
Beneluxa Initiative always
joint assessment will be

f countries to
cates a benefit, but
f hematopoietic stem
onsible for the

acute lymphoblastic leukaemia who receive CAR-T are likely to
determining comparative and cost effectiveness, the influence of this choice of treatment
carefully considered.

(°) Chimeric antigen receptor-engineered T-lymphocytes

NEWS ABOUT ACTIVITIES DOCUMENTS CONTACT

+ ...round documents

+ ...

...eluxa - statements

...Alzheimer's HTA

...therapies relative to Alzheimer's disease coming to the market. Recently the FDA in ...versy; a decision from EMA is not yet available.

...in cognitive scores or dementia scales are considered as the ...are not considered sufficient for demonstration of

...rvovec) for the treatment of ...iagnosis of SMA Type 1; or ...ene.

...of the product in each ...t of a reimbursement ...eXis/Novartis has stated ...three

...m

...is responsible ...contributes to this.

...portion of patients in paediatric acute lymphoblastic leukaemia who receive CAR-T are likely to ...receive stem cell transplants. In determining comparative and cost effectiveness, the influence of this choice of treatment pathway should be carefully considered.

01 October 2021 - Assessment of COVID-19 monoclonal antibodies is needed

The Beneluxa Initiative considers value assessments of medicines prior to reimbursement in their respective countries, to be essential in quantifying the potential added benefit of treatments relative to the price requested. This concept was reaffirmed in a recent joint statement with the Nordic Pharmaceutical Forum.[1]

Nach oben zum Inhaltsverzeichnis

04 June 202...

In recent years, efforts to enh... increased substantially. The need for in... concerning pricing, reimbursement and procurem... patients these issues are essential to solve in an effective a... platforms such as the Nordic Pharmaceutical Forum (NLF); constitut... Beneluxa Initiative; constituting of Belgium, the Netherlands, Austria, Ireland a... include the organisation of the international horizon scanning initiative (IHSI) as well as a... platforms. A number of strategic meetings have taken place where other international payers also ... the European Commission has launched a new Pharmaceutical Strategy for Europe highlighting similar issue... access to affordable medicines. The Strategy specifically refers to the strengthening of existing and potential new cro... collaborations.

Which drugs are of interest?

- Rare diseases
- High cost
- Mutual interest between countries

Beneluxa Assessment process

- Submission of concept dossier – feedback given.
- Final dossier submitted – Day 0.
- Assessment Group Review - Day 0-60.
- Draft report reviewed by the Dutch (WAR) and Belgian (CTG) expert committees.
- Report updated with comments and second draft sent for external consultation and to committees.
- Comments received and report finalized to be sent to Dutch and Belgian reimbursement committees and in Ireland the Drugs Group, HSE.
- End of HTA stage.

Negotiation Phase

- Agreement from all parties
- Once begun 120 days to agreement (Belgium)
- Joint negotiation between countries.
- Informed by the HTA report

Is Joint HTA possible?

PharmacoEconomics (2019) 37:627–630
https://doi.org/10.1007/s40273-019-00781-w

EDITORIAL

Beneluxa: What are the Prospects for Collective Bargaining on Pharmaceutical Prices Given Diverse Health Technology Assessment Processes?

James F. O'Mahony[1]

Published online: 8 March 2019

Is Joint HTA possible?

PharmacoEconomics (2019) 37:627–630
https://d 00781-w

EDIT

Costs

Utilities

Perspective

Discounting

Thresholds??

Beneluxa: What are the Prospects fo on Pha Assessment Processes?

James F. O'Mahony[1]

Published online: 8 March 2019

Zolgensma® (onasemnogene abeparvovec)

- ATMP – gene therapy
- IV infusion
- ~€2m/infusion

Novartis gene therapy

- Joint HTA
- Belgium, Ireland and the Netherlands
  - Pharmacoeconomic assessment (Model and budget impact) – Ireland (NCPE), Belgium.
  - Pharmacotherapeutic assessment – The Netherlands (ZIN)

Novartis

- Joint HT
- Belgium Netherlands
  - Pharm ment (Model and budge ICPE), Belgium.
  - Pharm ssment – The Nethe

Beneluxa Review Group Assessment Summary

Onasemnogene abeparvovec

for the treatment of patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene

April 2021

Is Joint HTA possible?

PharmacoEconomics (2019) 37:627–630
https://d 00781-w

EDIT

Costs

Utilities

Perspective

Discounting

Thresholds??

Beneluxa: What are the Prospects fo on Pha Assessment Processes?

James F. O'Mahony[1]

Published online: 8 March 2019

Success?

- Fascinating learning network
- Shared challenges, shared problem solving
- Not just about joint negotiation.
- More Joint HTAs, Zynteglo®, Libmeldy® and others…

BeNeLuxA et al.: the best is yet to come

by Yannis Natsis | Dec 28, 2019 | Universal Access and Affordable Medicines | Publications