



Zorginstituut Nederland

# Equitable access to ATMPs in the Netherlands: Impact of the EU regulation and the BENELUXA initiative on HTA

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## Presenter disclosure information

|                                                                                                                                                                                                      |             |
|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|-------------|
| Potential conflict of interest                                                                                                                                                                       | <b>None</b> |
| Potentially relevant company relationships for this meeting                                                                                                                                          | <b>None</b> |
| <ul style="list-style-type: none"><li>• Sponsorship or research funding</li><li>• Honorarium or other (financial) compensation</li><li>• Shareholder</li><li>• Other relations, namely ...</li></ul> | <b>None</b> |

# Introduction National Healthcare Institute

# National Healthcare Institute– what do we do?

National Healthcare Institute is an independent governmental body

- Acts as a referee and adviser

Key roles of ZIN

- Managing the basic health care package (health technology assessment and appraisal)
- Encouraging improvement in health care quality, affordability and accessibility
- Funding of the health care insurers
- Development of information standards
- Taking care of risk adjustment in the care sector

# Management of the basic health care package

The four package criteria

1. Effectiveness (knock out criterium) ← **EU collaboration**

*Is there evidence that a given treatment works?*

2. Necessity

*Is the disease serious enough? Is insurance the right instrument?*

3. Cost-effectiveness

*Is the ratio between the costs of a treatment and its results acceptable?*

4. Feasibility

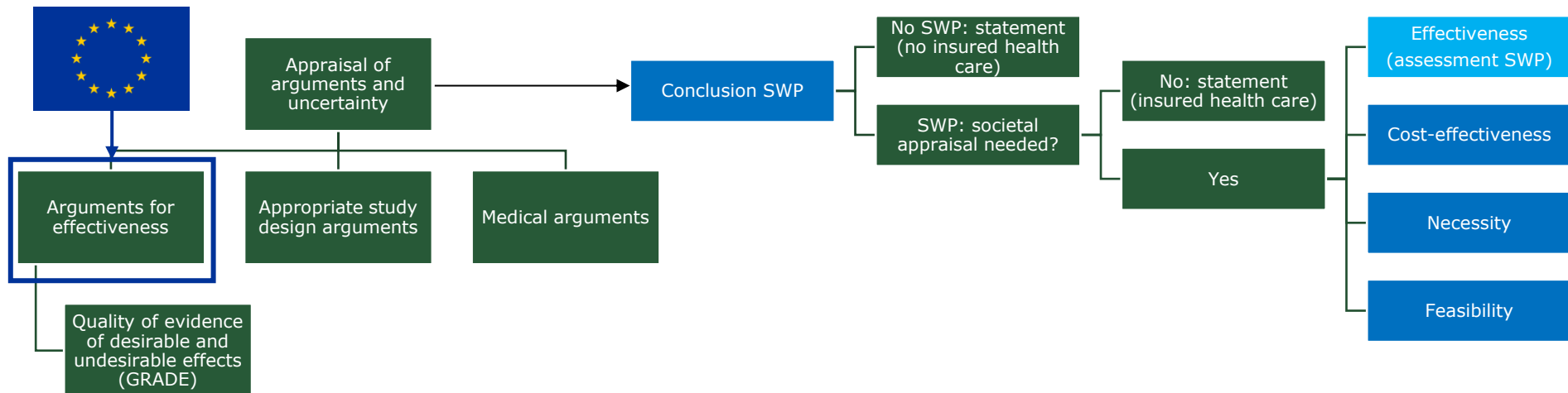
*Is inclusion of a given treatment in the package sustainable and feasible?*



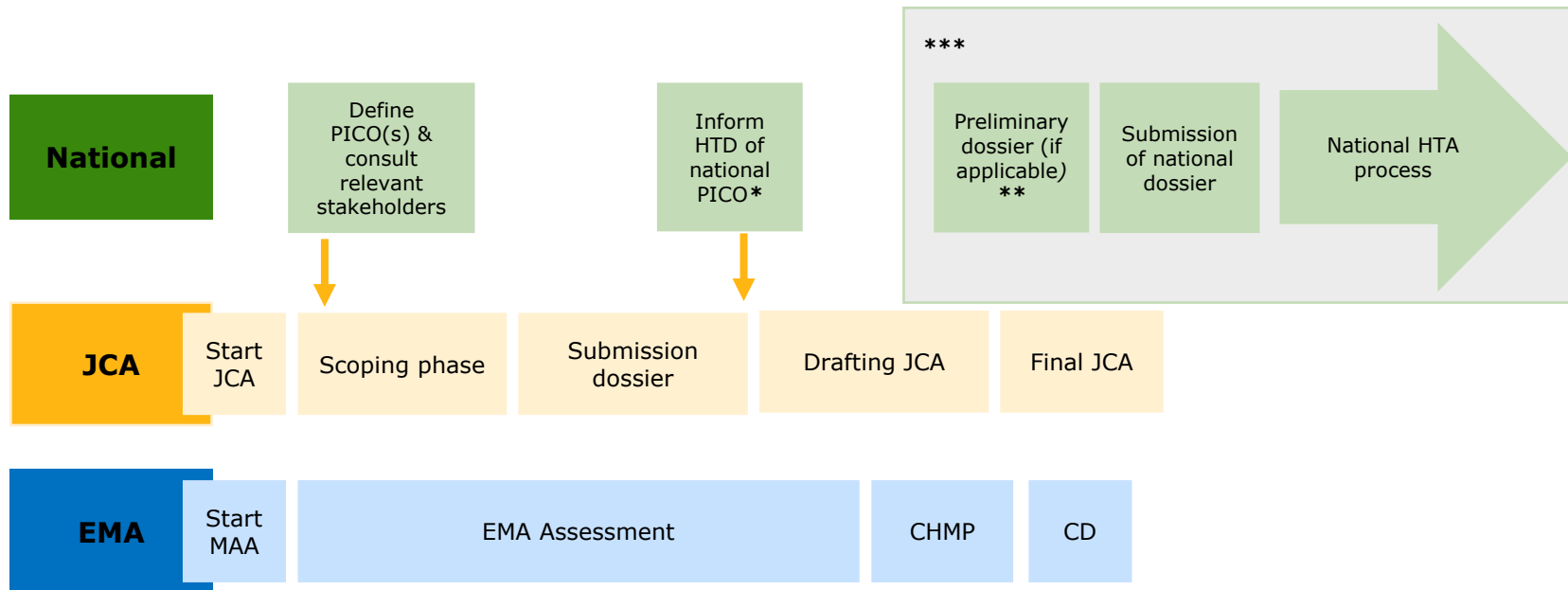
# General process – Evidence to decision

Assessment of “established medical science and medical practice”: statement

Appraisal package criteria: advise on reimbursement



# Dutch implementation of EU HTAR



**Abbreviations:** EMA=European Medicines Agency; CHMP=Committee for Human use of Medicinal Products; CD=Commission Decision on Marketing Authorization; HTD=Health Technology Developer; JCA=Joint Clinical Assessment; MAA=Marketing Authorization Application; PICO=Patient, Intervention, Comparator(s) and Outcome(s);

\*: only upon request by the HTD

\*\* : may include a renewed national scoping, this may especially be relevant when the HTD submits much later as the treatment landscape could have changed

\*\*\*: it should be noted that this figure presents the earliest timepoints possible for these steps, however, the exact timelines will depend on the national submission by the HTDs.

The background of the slide features a stylized Dutch flag with horizontal stripes of red, white, and blue. A small green vertical bar is positioned to the left of the title.

## Dutch assessment

- Must give due consideration to the JCA report
  - National appraisal can only start upon publication of JCA report
- National reimbursement dossier still required
  - For pharmaco-economic assessment
- ZIN writes (shortened) assessment reports
  - Referencing the JCA-report
  - Add: conclusion therapeutical value and reimbursement advice

# Changes and challenges on the implementation of the EUTHAR

- **Scoping starts much earlier**
  - Involvement of patient organizations, health insurers and clinical experts
  - Scope is not data-driven
- **ZIN can only take on assessor role for products that follow the 'lock procedure'**
  - (Very) high-cost in-hospital pharmaceuticals with high budget impact
- **JCA report is starting point of clinical assessment**
- **National scope may be renewed after submission of the national dossier**
  - Dependent on the launch strategy of the HTD or changes in therapeutic landscape



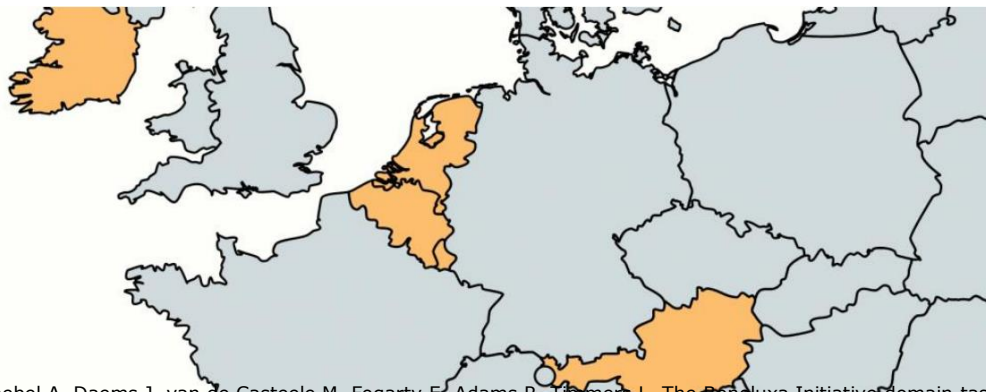
# BENELUXA initiative

# Beneluxa initiative

Beneluxa initiative is a partnership between 5 EU member states

- Belgium, the Netherlands, Ireland, Austria and Luxembourg
- Objectives are broader than the scope of the EUHTAR
- Focus is on new, complex and often very expensive medicines for a (very) small group patients or severely sick people.
- Assessments conducted within Initiative automatically qualify as national assessments

**The main goal is to improve  
accessibility and affordability of  
medicines**



# Areas of cooperation

- **Horizon scanning**

- Identification of emerging health technology
- Select those for joint assessment
- E.g. International Horizon Scanning Initiative (ISHI)

- **Health Technology Assessments**

- Comparative effectiveness, cost-effectiveness and budget impact

- **Pricing and reimbursement**

- Sharing information on the cost build-up of pharmaceuticals

- **Information sharing and policy exchange**

- Exchange information on medicinal product policy (such as insured package advice), the assessment of innovative care technology and best practices with each other

## Example

| Name pharmaceutical | Active substance         | Therapeutic area        | Year      | Type of HTA-collaboration                                                                                                                                                                                                                                                           |
|---------------------|--------------------------|-------------------------|-----------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| SPINRAZA            | Nusinersen               | Spinal muscular atrophy | 2018      | <ul style="list-style-type: none"> <li>-RIZIV-INAMI used assessment report by ZIN</li> <li>-Other HTA organisations used the final report</li> <li>-Joint negotiation with Belgium &amp; the Netherlands -&gt; successful</li> </ul>                                                |
| ZOLGENSMA           | Onasemnogene abeparvovec | Spinal Muscular Atrophy | 2020/2021 | <ul style="list-style-type: none"> <li>-Joint writing by Belgium, the Netherlands and Ireland</li> <li>- DSVS acted as external referee for RIZIV-INAMI</li> <li>-Joint negotiation with Belgium, the Netherlands and Ireland -&gt; successful</li> </ul>                           |
| ZYNTEGLO            | Betibeglogene autotemcel | Beta thalassemia        | 2021      | <ul style="list-style-type: none"> <li>-Joint writing by Belgium &amp; the Netherlands</li> <li>-NCPE acted as external referee for RIZIV-INAMI</li> <li>-Joint negotiation with Belgium &amp; the Netherlands -&gt; manufacturer withdrew application for reimbursement</li> </ul> |

# Implications for ATMP

# Current challenges

- Evidence is scarce (e.g., single arm trials)
- Number of patients is often low
- Very expensive
- Preparation reimbursement dossier takes times

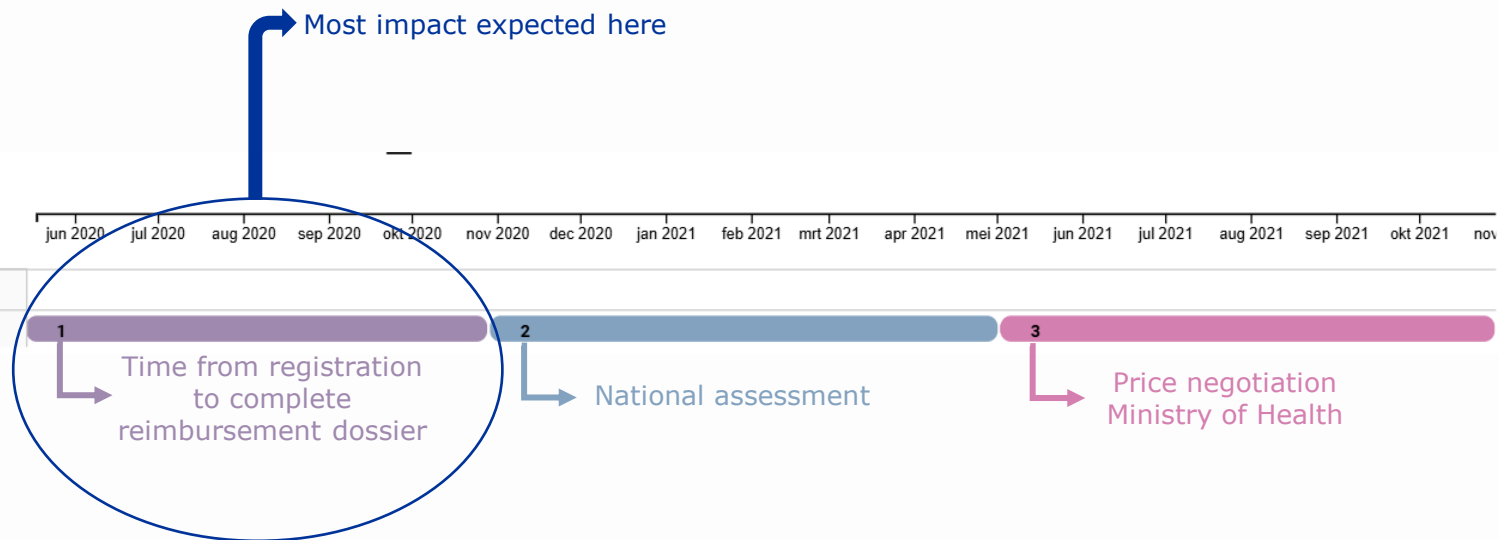


## Doorlooptijden

^ Alles samenvouwen

[-] Spinale musculaire atrofie (SMA, spierziekte)

Zolgensma





## How can EU HTAR and Beneluxa enhance reimbursement of ATMPs?

- **Early EU HTAR assessment parallel to EMA registration**
  - Sharing scope of the assessment prior to registration
    - HTD can use this for reimbursement dossier preparation
- **Improving quality of evidence**
  - e.g., indirect comparison for single arm trials
- **Reducing duplication in national process**
- **Joint assessment on cost-effectiveness and collaborations on price negotiations**