

# Marketplace of PPRI Queries

Exagamglogene autotemcel (Exa-cel, Casgevy®)

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## General information

- **Pricing and reimbursement information** for Exagamglogene autotemcel (Casgevy®)
- Launched in **October 2024** by Austrian MoH
- Responses from **17 countries**  
(CY, CH, CA, UK, SE, CZ, ES, PT, PL, BG, IT, UKR, ISR, BRA, SAU, FR, AT)
- Information on: Trade name, company, **price, reimbursement status (Y/N), reimbursement conditions** / limitations / eligible patient group, expected patient number, patients treated or in preparation for treatment, optional: further info

## Purpose of the query

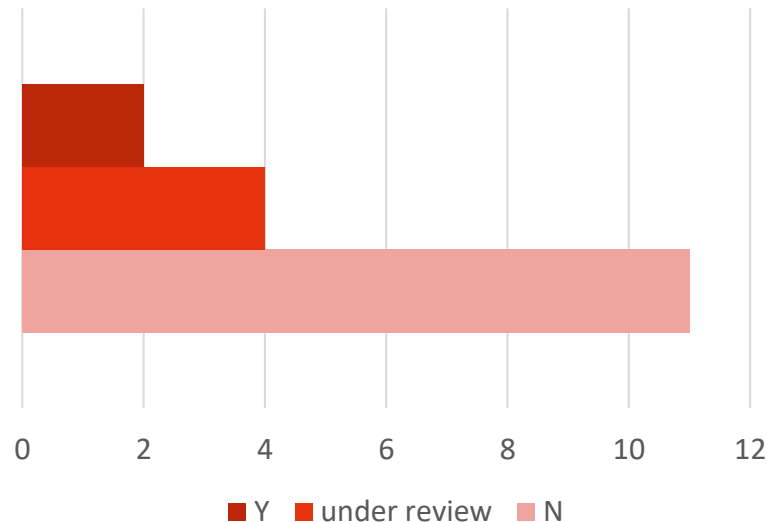
- Information for the **Austrian Appraisal Board**
  - **national assessment and appraisal process** for selected (“specialized and high-priced”) medicines used in hospitals and at the interface with the outpatient sector
  - national expert committee issues **Austrian-wide recommendations** in an expert opinion paper (including relative additional benefit in relation to economic aspects, recommendations regarding use, application criteria for use)

## Results for pricing and reimbursement status

- **Pricing information:**

- UK: list price is £1.651 million  
(but additional confidential commercial arrangement has been agreed)
- FR: unnegotiated (maximum) price is €1.9 million (early access reimbursement) with mandatory clawback after negotiation

- **Reimbursement status:**



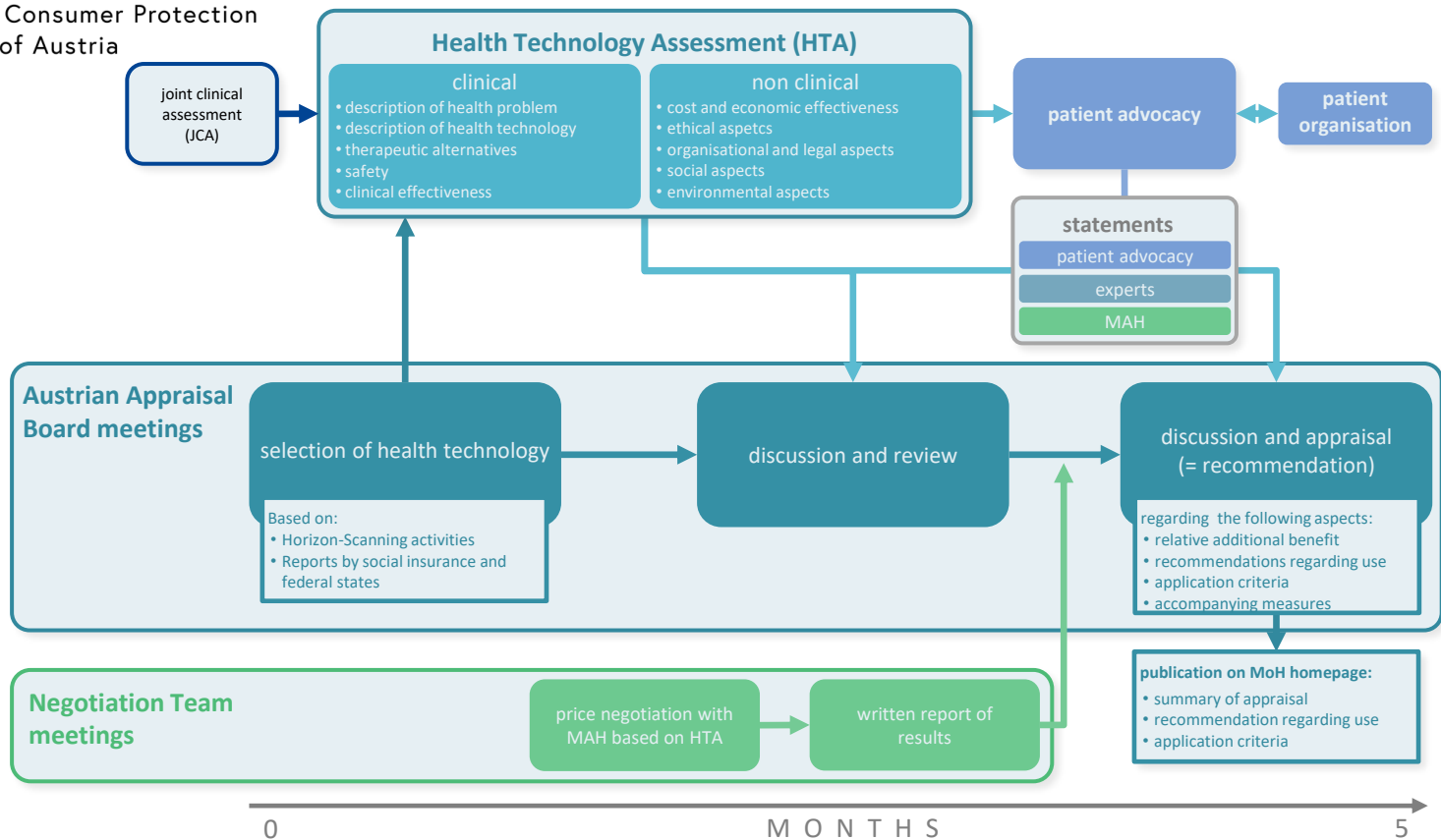
## Results for reimbursement conditions

- **Reimbursement conditions:**
  - UK: **Managed access** for patients with **transfusion-dependent  $\beta$ -thalassaemia (TDT)** in patients  $\geq 12$  years when haematopoietic stem cell transplant (HSCT) is suitable but human leukocyte antigen (HLA)-matched related donor is not available; **further data collection** to address uncertainties in evidence base, **re-assessment of cost-effectiveness planned** in the future
  - FR: **Early Access** for patients (age 12-35 years, when HSCT is suitable but HLA-matched related donor is not available) with **TDT** or **severe sickle cell disease** (with persistent vaso-occlusive crises requiring hospitalisation, despite hydroxycarbamide therapy)

## Impact and further development of queries

- **International perspective** on prices, reimbursement status & respective conditions
  - included in national **Health Technology Assessment**
  - beneficial for national **pricing negotiations**
- **Further development** of queries launched for the Austrian Appraisal Board
  - Price, reimbursement status, reimbursement conditions (MEA)
  - SoC therapies, indication, (expected) patient numbers

## Workflow of an appraisal process



**MAH:** Marketing authorization holder

**MoH:** Ministry of Health (Federal Ministry of Labour, Social Affairs, Health, Care and Consumer Protection)

**Zielsteuerung-Gesundheit**

Bund • Länder • Sozialversicherung

## Contact – Office of the Austrian Appraisal Board

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