



# The Union List of Critical Medicines

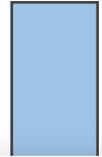
Medicines we need the most?

Joint PCWP-HCPWP meeting 3-4 February 2026,  
EMA

# What does it look like?

ATC level 5	<b>ATC description</b> The Anatomical Therapeutic Chemical code: a unique code assigned to a medicine according to the organ or system it works on and how it works. The classification system is maintained by the World Health Organization (WHO).	Route of administration	Date of inclusion
A - Alimentary tract and metabolism			
A02B - Drugs for peptic ulcer and gastro-oesophageal reflux disease (GORD)			
A02BC05	ESOMEPRAZOLE	intravenous use	1 December 2023
A03B - Belladonna and derivatives, plain			
A03BA01	ATROPINE	intramuscular, intravenous, subcutaneous use	1 December 2023
A03F - Propulsives			
A03FA01	METOCLOPRAMIDE	intramuscular, intravenous, subcutaneous use	1 December 2023

# Addition of indications for rare disease



<b>ATC level 5</b>	<b>The Assigned works main</b>
	A02B - I
<b>Frequent use</b>	ESOMEPI
	A
A03BA01	ATROPINE

- ▶ Monographie complète
- ▶ Forme(s) pharmaceutique(s)/Composition
- ▶ Classifications/pharmacologie
- ▶ Médicaments virtuels/Génériques
- ▶ Recommandations
- ▶ Renseignements administratifs
- ▶ Conservation
- ▶ **Indications**
- ▶ Non indications
- ▶ Posologie(s)/administration
- ▶ Contre-indications
- ▶ Mises en garde et précautions d'emploi
- ▶ Non contre-indications

**ESOMEPRAZOLE ALM 20MG GELULE**  
**ESOMEPRAZOLE ALMUS 20 MG, GELULE GASTRO-RESISTANTE**  
Fiche(s) d'identité interopérable  
Historique des références officielles

**Maladie rare** Non

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**ZOLLINGER-ELLISON SYNDROME** SMR Niveau IMPORTANT  
CHEZ L'ADULTE  
TRAITEMENT CURATIF

\* Adultes

Ce médicament est indiqué dans le traitement du syndrome de Zollinger-Ellison.

**SMR de l'indication** NIVEAU IMPORTANT 30/10/1999

**Référence(s) officielle(s)** Rectificatif AMM française 02/09/2025

**Maladie rare** Oui - Lien Orphanet : [Syndrome de Zollinger-Ellison](#)

intramuscular, intravenous, subcutaneous use

1 December 2023

# Products used in rare diseases (off-label use not considered)

- 98 active substances on the list for a frequent use, with at least one indication for a rare disease
- For 117 rare conditions
  - Rare cancers (50)
  - Rare infectious diseases (22)
  - Rare epilepsies (13)
  - Inflammatory-immunology (9)
- And 25 active substances with indication(s) specific to rare diseases
- For 29 rare conditions
  - Rare cancers (8)
  - Haemophilia (5)
  - Rare infectious diseases (4)
  - Inflammatory-Immune (4)

# Some singularities

ATC level 5	ATC description	Year marketing authorisation
R05CB13	DORNASE ALFA (DESOXYRIBONUCLEASE)	2005 (specific)

Cystic fibrosis: most useful products not on the list (disease modifiers, life-prolonging) (Kaftrio® ...)

ALS: Riluzole on the list, despite limited clinical benefit (2-month life extension)

Multiple Myeloma: Thalidomide is on the list, Lenalidomide is not, and yet more often used and more effective, less toxic than thalidomide

Myasthenia gravis: neostigmine is on the list, and yet patients prefer pyridostigmine

Imiglucerase alfa (Gaucher type 1&3) on the list: velaglucerase? eliglustat? miglustat? Which is best?

Alglucosidase alfa (Myozyme®): life-saving for early onset Pompe disease, but not on the list

Anti-haemophilia products on the list, but none for Willebrand disease type 2-3

Obsolete?

HIV: all products on the list are outdated, with high prevalence of viral resistance, when more recent classes are missing (active against resistant strains, more powerful, less toxic, easier to take)

Acetylcysteine: anti-cough medicine. Needed? Or is it for other uses (mushroom poisoning)?

# Are we satisfied with the list?

## Critical Medicines Act: the right measures but applied to the wrong medicines?

- *Our first impression: a list with the **appearance of rationality**, seemingly evidence based, but in fact more **a political construct***
  - *Decision not to allocate resources to screen 12,500+ active substances*
  - *Necessity to have 15 MS agreeing*
    - *Why would MS agree a medicine should be **available at all times**, when the national decision in that MS was to not reimburse it?*
- *Double concern*
  - *on the list: are they the ones **patients/citizens need the most**? What if not?*
    - *If the right one(s) for your disease on the list: important to confirm*
  - *not on the list but patients / clinicians think they should be: how to **have them considered**?*
    - *When you ask patient organisations: all medicines are critical ones*
- *286: many already, can't have many more*
- *An analysis with the Académie Nationale de Pharmacie (France), other academies, other patient organisations, learned societies?*
- *Webinar for EURORDIS members and Federations 4 March*

**Thank you!**

# justification

imiglucerase alfa	velaglucerase	eliglustat
Cerezyme® - 1997	Vpriv® - 2010	Cerdelga® - 2015
patients who have type <b>1 Gaucher</b> disease (95%), which does not affect the nerve cells, or type <b>3 Gaucher</b> disease, which progresses slowly and affects the nerve cells	patients who have type-1 Gaucher disease, the type that usually affects the liver, spleen and bones.	long-term treatment of type 1 Gaucher disease in adults and in children
From hamsters	Recombinant – useful if interruption in the production of imiglucerase cf HAS (so there is an alternative)	CYP2D6 contra-indications
1 <sup>st</sup> line	1 <sup>st</sup> line	2 <sup>nd</sup> line, more effective than miglustat (Zavesca®)
Major shortage 2009 x 2 years		