Navigating Compassionate Use in the Netherlands: Opportunities and challenges from a company perspective

Dutch Medicines Days / RSNN

Johnson&Johnson
Innovative Medicine

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Disclosure of speaker's interest
Employee of **Johnson & Johnson Innovative Medicine NL**
Member **RSNN Advisory Board**
Chair **VIG RA Group** (trade association)
Member **NVFG RegNed Board** (professional association)

Why is compassionate use important?

Our Credo

The values that guide our decision-making are spelled out in Our Credo. Put simply, Our Credo challenges us to put the needs and well-being of the people we serve first.

We believe our first responsibility is to the patients, doctors and nurses, to mothers and fathers and all others who use our products and services. In meeting their needs everything we do must be of high quality. We must constantly strive to provide value, reduce our costs and maintain reasonable prices. Customers' orders must be serviced promptly and accurately. Our business partners must have an opportunity to make a fair profit.

- Patients are waiting
- The Dutch healthcare system fortunately offers several options

Which early access pathways do we have in NL?

Access Pathway		Registered for this indication?	State of Science and Practice?	Registered for other indication?	Treatment covered by reimbursement?
Regular reimbursed treatment		✓	✓		Insurance company
Early access	*Free of charge during lock*	✓	*Uncertain*		*Pharma company*
	Conditional approval (orphans, conditionals)	✓	*Uncertain*		*Insurance company*
	Orphan Drug Access Protocol	✓	*Uncertain*		*Insurance company*
	Drug Access Protocol	✓	*Uncertain*		*Pharma + Insurance company*
	Off-label indication	X	✓	✓	*Insurance company*
	Off-label indication (compassionate use)	X	X	✓	*Pharma company*
	Compassionate Use (MEB)	X	X	X	*Pharma company*
	Doctor's Declaration (IGJ)	X	X	X	*Pharma company*
Participation in clinical study		X	X	X	Pharma company

https://www.hollandbio.nl/nieuws/early-access-lets-go-dutch-2/

How is J&J managing early access on a global level?

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About us | Our innovation | How we work | Careers | Newsroom

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Pre-approval access

Around the world, access to investigational medicines outside of a clinical trial is named in various ways. We use the term “Pre-Approval Access”, which is also commonly called, “Compassionate Use”. For more on common terminology, click here.

Our pre-approval access policy

Read more →

Our investigational medicines with PAA Programs

Read more →

Resources for patients, caregivers and providers

Read more →

What is CompAC?

Read more →

Pre-approval access by the numbers

Read more →

When is J&J allowing early access?

When will a decision about a PAA request from J&J Innovative Medicine be available?

We commit to providing a decision as quickly as possible, ideally, within **5 to 10 business days** of receiving all required medical information.

1. The patient must have a **serious or life-threatening disease or condition**
2. There must be an **unmet medical need**, **alternative therapies are not available**, or the patient must have **exhausted all such alternative therapies**.
3. The patient is not eligible or cannot participate in a **clinical trial**. In assessing the eligibility of a patient for potential PAA, **preference will be given to clinical trials, then pre-approval access programs, and then single patient access**.
4. **Sufficient scientific evidence** to demonstrate that the benefits of the investigational medicine outweigh the risks.
5. Providing PAA will **not jeopardize** the initiation, conduct, or completion of clinical investigations and the **overall development program** to support registration of the product.
6. PAA must be permitted by, and **run in accordance with, applicable laws**.
7. The treating physician making the request is **licensed and qualified** to administer the investigational medicine and agrees to comply with Janssen requirements and local regulations governing PAA and adhere to applicable laws and regulations.

How do we as J&J NL start early access?

Inspectie Gezondheidszorg en Jeugd
Ministerie van Volksgezondheid, Welzijn en Sport

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Artsenverklaring voor leveren geneesmiddel zonder handelsvergunning

Dit formulier is nodig om een aanvraag te kunnen doen voor het leveren van geneesmiddelen zonder handelsvergunning. Zie verder 'Leveren op artsenverklaring'.

Download: Artsenverklaring voor leveren geneesmiddel zonder handelsvergunning

Formulier | 22-03-2022 | PDF-document | 142.98 KB | 2 pagina's

CBG MEB
MEDICINES EVALUATION BOARD

Policy document

Compassionate Use Programme

MEB 49

December 2024

Overzicht goedgekeurde compassionate use programma's

Overzicht inklappen

2025

2024

Balversa (erdafitinib)

2023

Omjjara

Company name: GlaxoSmithKline B.V.
Indication: Omjjara is indicated for the treatment of disease-related splenomegaly or symptoms in adult patients with primary myelofibrosis, post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis and moderate to severe anaemia who have previously been treated with a JAK inhibitor (ruxulotinib or fedratinib) and for whom there are no satisfactory alternative treatments.
Date of approval: 10 november 2023

Talquetamab

Company name: Janssen-Cilag B.V.
Indication: Treatment of adult patients with relapsed or refractory multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody and who have exhausted all available treatment options accessible as local standards of care and who have demonstrated disease progression on the last therapy.
Date of approval: 14 juli 2023

Some challenges

CUP (MEB)

- For specific conditions
- Submit PSUR after 1 year
- Data collection allowed
- Ends at EMA approval
- Approval within 6-8 wks

Continuation of CUP?

- For former CUP patients as they must be treated until launch?
- For new patients too?
- Advantage: 1) less discussions with company HQs and 2) data collection can continue

Free of charge during lock?

- No data collection

Docter's Declaration (IGJ)

- For more broad conditions
- No need to intent to submit a MAA
- Single patients or cohorts
- 1 year; extension possible
- Approval within 1-3 days

Additional risk minimization materials (MEB)

IMP (IGJ)	TAV (IGJ)
Deliver IMP until commercial pack is available	Deliver pack from other EU country e.g. Germany

Some other challenges

- **Heterogenicity Europe**
  - pathways vary between countries –> no equal access
  - complex for company headquarters
- **Data collection** -> CUP/DD run in parallel with clinical trials -> wish to pool RWD
- **Publicly funded early access** (a.o. FR, IT, BE)

Conclusion

- Several pathways for compassionate use available in NL -> flexibility

- Extension of CUP until reimbursement/launch
  - get approval from company HQs
  - data collection
  - support HTA & pricing decisions

Collaboration *between healthcare providers, regulators, patient representatives and pharmaceutical companies is* ***essential*** *to* ***ensure timely access*** *to promising therapies while maintaining patient safety and regulatory compliance.*

More info

- https://english.cbg-meb.nl/topics/mah-compassionate-use-programme
- https://www.igj.nl/zorgsectoren/geneesmiddelen/beschikbaarheid-van-geneesmiddelen/leveren-op-artsenverklaring
- https://www.jnj.com/innovativemedicine/pre-approval-access
- https://www.hollandbio.nl/nieuws/early-access-lets-go-dutch/

Thank you

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