FROM UNCERTAINTY TO CONFIDENCE

THE FAST TRACK FRAMEWORK FOR RARE DISEASE ACCESS IN THE NETHERLANDS

Co-developed by:
Empowered By Us
Alexion Netherlands

Draft for consultation ahead of the October 2025 Roundtable

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Executive Summary

Rare disease patients in the Netherlands wait far too long for access to approved treatments. While therapies are available elsewhere in Europe, Dutch patients often face years of uncertainty due to lengthy procedures, stalled negotiations, and dossiers piling up in the system. Families are increasingly resorting to crowdfunding for care abroad, while clinicians are left frustrated that they cannot treat patients who could benefit from these therapies.

This is not a marginal issue – it is a systemic failure. The Fast Track framework has been co-developed by Empowered By Us (rare disease patient leadership), Alexion Netherlands, and HTA experts, and will be stress-tested with clinicians, pharmacists, patients, payers, and policymakers in October 2025. It is designed as a complementary pathway, not a replacement: a patient-first, policy-anchored framework that manages uncertainty through shared responsibility.

At its core, Fast Track introduces a confidence-based tiered model that links patient relevance, evidence, and system fit. This replaces a system designed for mainstream medicines, which is effective at controlling costs in large populations but structurally ill-suited to rare diseases. Fast Track provides a pathway that delivers earlier access with managed risk, anchored in patient relevance.

Mandates and Policy Context

The proposed Fast Track a structured, sustainable, and proportionate approach to evaluating and managing treatments for rare and ultra-rare diseases where uncertainty is high. It builds directly on the 2024 Cabinet letter from Minister of Health Fleur Agema[1], which committed to developing a fast-track procedure for a select group of medicines, ensuring that patients in high need do not have to wait unnecessarily for clarity on reimbursement, and on the 2024 Paulusma/Bushoff motion[2] mandating faster access routes for orphan drugs. It aligns with priorities expressed by the Dutch Association of Innovative Medicines (VIG)[3], and recent policy developments including the 2025 evaluation of Conditional Admission[4] and the Cabinet's Vision on Biotechnology 2025–2040[5].

Next Steps

The framework will be stress-tested with policymakers, clinicians, pharmacists, patients, and payers at the October 2025 *Policy & Perspectives* Roundtable. Based on these insights, it will be refined and validated through a co-designed pilot in 2026, ensuring practical feasibility and national adoption first, with learnings feeding into the EU debate.

Fast Track gives the Netherlands the opportunity to move from a reactive, delayed system to one that designs for equity and inclusion from the start: positioning the country as a frontrunner in rare disease policy at both national and European levels.

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[1] Minister of Health (Fleur Agema) Letter. (2024, October 4). Tweede Kamer der Staten-Generaal. https://www.tweedekamer.nl/kamerstukken/brieven_regering/detail?did=2024D36824&id=2024D36824. Accessed July 2025
[2] Motion by members Paulusma and Bushoff. (2024, June 12). Tweede Kamer der Staten-Generaal. https://www.tweedekamer.nl/kamerstukken/moties/detail?id=2024Z10302&did=2024D24327. Accessed July 2025
[3] Vereniging Innovatieve Geneesmiddelen (VIG). (2024). VIG Vision 2024. VIG (Weesgeneesmiddelen). https://www.weesgeneesmiddelen.nl/weesgeneesmiddelen/weesgeneesmiddelen/home/3351/VIG-Weesgeneesmiddelen-Visiedocument.pdf. Accessed July 2025
[4] ZN Orphan drug access protocol/ODAP Reforms. (2025). Zorgverzekeraars Nederland. https://www.zn.nl/dossiers/orphan-drug-protocol/. Accessed July 2025
[5] Dutch Cabinet Vision on Biotechnology 2025-2040. (2025). Rijksoverheid. https://open.overheid.nl/documenten/a83e3ff3-ac9e-45be-8c8a-ace92a9e7e3e/file. Accessed July 2025

Fast Track – At a Glance

Purpose	**Faster, fairer, sustainable, and efficient access for rare disease patients**
Focus	Treatments for rare and ultra-rare diseases
Core Components	Patient Relevance • Evidence Generation • System Fit • Shared Governance
Assessment Model	Tiered confidence system: High • Moderate • Low
Outcome	Proof-of-concept pilot in 2026 → Pathway to NL adoption first with a protocol/orphan drug arrangement led by patients and clinical experts • Standard of Care

The Challenge: Why Current Systems Fail Rare Disease Patients

> *"The biggest barrier for rare disease patients today is not science, but the system."*

Challenges In Orphan Drug Access

Orphan drugs treat conditions affecting fewer than 5 per 100,000 people (fewer than 2 per 100,000 in ultra-rare cases). In the Netherlands, this translates to patient populations of fewer than 900 or 360 individuals, respectively.[6] Small populations create evidentiary and economic constraints that conventional assessment and reimbursement processes are not built to handle.

For patients, this means delays are not just administrative – they can result in irreversible disease progression, or loss of independence. Families and clinicians are left in limbo, despite therapies already being authorised at the European level.

The rarity of these diseases makes large-scale randomised trials impractical. Evidence often comes from small cohorts or case series, increasing uncertainty. Current methods (e.g., GRADE) apply general standards that fit poorly in this context, adding administrative complexity – especially for in-hospital therapies that follow non-standard routes.

Current Pathways In The Netherlands

PATHWAY	DESCRIPTION	PATIENT IMPACT
WGA (Orphan Drug Arrangement)	Managed entry based on agreements on appropriate use and monitoring. Still part of the Fast Track.	Delays from rigid thresholds and sometimes unrealistic discounts.
ODAP (Orphan Drug Access Protocol)	Pilot protocol, designed to support structured data collection.	Participation without guaranteed access due to limited capacity.
CONDITIONAL ADMISSION (VT)	Temporary reimbursement for up to seven years, with re-evaluation based on follow-up evidence. Not appropriate for ultra rare diseases.	Delays in availability; patients wait years for effective treatment.
SLUIS (Lock System)	Medicines with projected annual cost > €10 million are assessed through the Sluis to balance value and price.	For orphan drugs, Sluis procedures average around 1,000 days[7], delaying availability to patients.

[6] Hegger, I., & De Vries, C. (n.d.). Databank voor zeldzame aandoeningen (360110001/2007). RIVM. https://www.rivm.nl/bibliotheek/rapporten/360110001.pdf. Accessed July 2025

[7] Publicly available industry analyses have reported that, for orphan drugs, procedures under the Dutch Sluis average around 1,000 days before patient access is granted. https://www.astrazeneca.nl/geneesmiddelen/zeldzame-ziekten1.html. Accessed September 2025

System Gaps

Across these pathways, there is no standardised, proportionate route tailored to the realities of orphan drugs. The result:

- Dossiers often delayed or not accepted into the system, leaving patients in limbo.
- Price negotiations frequently break down under unrealistic discount expectations.
- Conditions for reimbursement applied inconsistently, creating uncertainty.
- Treatments offered in other countries first, while Dutch patients wait.
- Parliament has already mandated faster solutions (Paulusma/Bushoff motion), yet no concrete plan delivered.

Result: Patients continue to wait, clinicians remain frustrated explaining delays, and the Netherlands is falling behind other EU countries in the availability of treatments for patients with rare disease, which undermines both patient outcomes and the country's position in biotech leadership.

Paradigm Shift – From Risk-Sharing to Responsibility-Sharing

For years, the debate on the availability and reimbursement of orphan drug has centred on risk-sharing, shifting financial and evidentiary uncertainty between payers, clinicians, and manufacturers. While well-intentioned, this model has proven insufficient. It manages risk transactionally rather than structurally and has not delivered faster or fairer outcomes for patients.

Fast Track introduces a new paradigm: responsibility-sharing. Instead of passing risk between parties, stakeholders take collective responsibility for timely and equitable availability of treatments, each within their distinct role.

> *"This is not about equal power, but balanced responsibility, anchored in patient and policy leadership."*

Shared Responsibilities In Practice

- **Patients** co-define outcomes that matter and participate directly in governance.
- **Clinicians and pharmacists** design and lead treatment protocols, including appropriate use, to optimise outcomes.
- **Insurers** provide oversight and are engaged to ensure affordability, sustainability, and system fit.
- **VWS and ZIN** arbitrate thresholds and safeguard sustainability.
- **Industry** contributes evidence transparently.

Patient Expertise As Infrastructure

Patient expertise is not tokenism - it is infrastructure.

- **At the individual level**, patients define outcomes that matter.
- **At the community level**, patient organisations shape care pathways and provide structured input.
- **At the policy level**, patient leaders co-govern criteria and oversight, ensuring accountability.

By embedding responsibility-sharing and patient expertise at the core, Fast Track moves beyond transactional risk-sharing to a structural model of co-ownership: credible for policymakers, usable for clinicians, and equitable for patients.

Fast Track Framework – How It Works

Fast Track introduces a tiered confidence system that links access decisions to patient relevance, strength of evidence and system fit. In rare disease, further studies may take years; therefore, "evidence" here also includes micro-level or targeted real-world insights that can be generated while patients receive treatment in the Netherlands.

From principle to mechanism

Responsibility-sharing is operationalised through:

- A confidence-based tiering system (Green = HIGH, Amber = MODERATE, Grey = LOW/DEVELOP), that replaces risk-averse decisions with proportionate ones grounded in evidence and patient relevance.
- When confidence in the data, expertise and organization of care is high, fewer mitigating measures are needed than when there is less confidence. With low confidence, all parties will need to actively collaborate to address uncertainties and risks through development of protocols and additional agreements.
- A co-designed pilot in 2026, with patients, clinicians, and pharmacists shaping outcomes and protocols, and insurers actively engaged to safeguard financial feasibility and sustainability. Results will feed into a 2026 adoption decision and support Standard of Care.

At A Glance: Confidence Tiers (Rapid Review and Triage)

Tier	Definition	Patient Impact	System Action
***HIGH* (Green)**	High confidence. Evidence and outcomes sufficient.	Broad availability confirmed.	Adopt into the basic package.
***MODERATE* (Amber)**	Moderate confidence. Evidence promising but incomplete.	Availability under defined conditions (time-bound, scope-limited, monitored).	Conditional adoption with structured monitoring and additional data collection.
***LOW/DEVELOP* (Grey)**	Low confidence. Evidence insufficient or unclear.	Availability under conditions decided upon by all relevant parties together and led by clinical and patient expertise.	Develop a protocol with appropriate use guidance, targeted data collection or micro-level evidence.

*** See Annex 1 for full scoring framework and criteria thresholds*

Core Criteria in the Rapid Review

The confidence tiers are determined by applying six criteria that reflect both patient priorities and system needs:

1. **Patient Relevance** – Outcomes co-defined with patients.

2. **Necessity** – Degree of unmet need and availability of alternatives.
3. **Effectiveness** – Strength of evidence for meaningful patient outcomes.
4. **Cost-Effectiveness** – Value when rarity and patient priorities are factored in.
5. **Feasibility** – Ability to deliver equitably to eligible Dutch patients.
6. **Budget Impact** – Proportionality of costs relative to population and need.

Methods and Technical Details

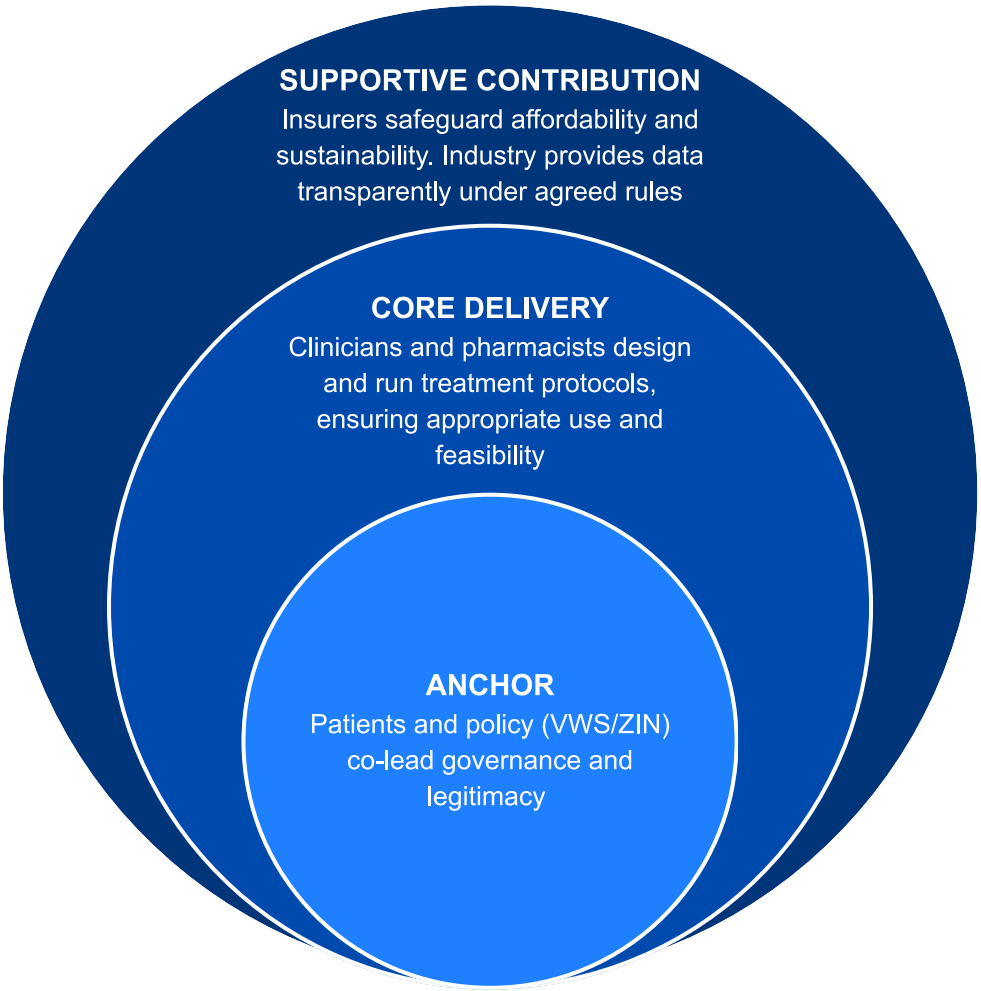
The full scoring framework, including confidence levels, thresholds, and methodological references, is provided in Annex 1.

Stakeholder Roles And Responsibilities

Fast Track depends on shared responsibility. Governance must remain anchored in patients and policy to ensure legitimacy and credibility, while clinicians, pharmacists, payers, and industry contribute their expertise in defined ways.

- Patients Co-define outcomes that matter, embed patient relevance into the criteria, and co-lead governance and oversight.
- VWS and ZIN Arbitrate thresholds, determine system fit and safeguard sustainability, ensuring Fast Track remains aligned with national policy priorities.
- Clinicians and Pharmacists Design and lead treatment protocols, including appropriate use, collect real-world evidence, and ensure feasibility in daily practice.
- Insurers Safeguard affordability and sustainability. In early pilots they may act in observer role but ultimately guarantee financing and responsible use.
- Industry Contributes transparent data and evidence.

Shared Governance Map (Principle-Based)



Pilot as Proof-of-Concept

The Fast Track framework will only be credible if it can be shown to work in practice. A pilot phase is therefore essential to show that all parties can work together effectively.

Purpose

The pilot will demonstrate whether Fast Track can deliver feasible and sustainable outcomes in practice with a protocol and/or an orphan drug arrangement, proving the framework's credibility for national adoption. Because it can be implemented within the current legal framework, testing can begin as soon as stakeholders agree and commit on scope, governance and deliverable.

Design

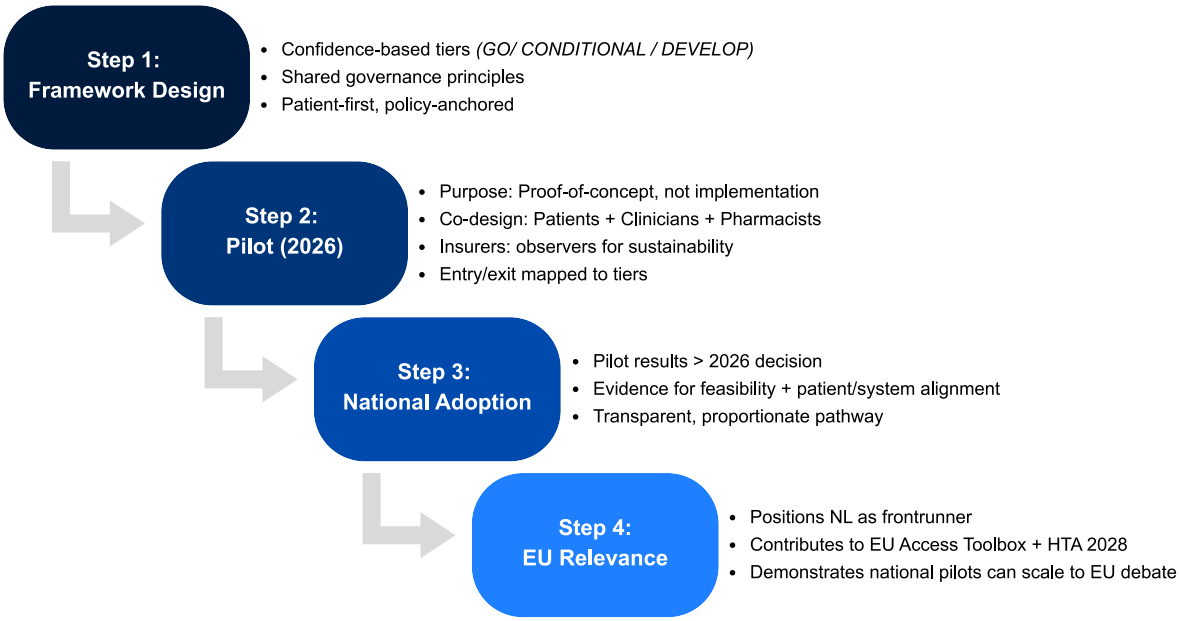
- **Co-design:** Patients, clinicians, and pharmacists will jointly shape scope, outcomes, and protocols.
- **Insurer:** Engaged to ensure affordability and sustainability from the start.
- **HCP collaborations**: Ongoing work with clinicians and pharmacists provides a foundation.
- **Transparency:** Entry and exit rules are mapped in the confidence assessment during rapid review and triage.

Use of Results

- Feasibility of the framework in day-to-day practice.
- Alignment between patient relevance, clinical outcomes, and system thresholds.
- The conditions under which proportionate adoption is sustainably solidified in a protocol and/or an orphan drug arrangement.

Ultimately, the Fast Track must be established whether a treatment for patients within the Dutch healthcare system meets the Standards of Science and Practice.

Findings will be shared with Parliament, VWS, ZIN, and patient organisations as a structured proof-of-concept. They will also inform the Netherlands' contribution to the EU debate on early and equitable access.



"The pilot is the credibility bridge: it translates framework principles into tangible evidence, ensuring Fast Track moves from theory to national adoption, and positions the Netherlands as a frontrunner in rare disease policy at both national and European levels."

Conclusion And Calls To Action

Fast Track provides the Netherlands with a credible, sustainable, and proportionate route to move from delay and deadlock to faster, fairer availability and reimbursement of treatments for people with rare diseases. It complements existing procedures by correcting a system misaligned with the needs of rare disease patients, replacing delay and deadlock with confidence-based, patient-first choices linked to evidence and system fit. The Netherlands is already falling behind other EU countries in rare disease access. Without reform, this gap will widen, weakening its position in biotech leadership.

The framework directly responds to the Paulusma/Bushoff motion (2024) for faster orphan drug access, aligns with the 2025 evaluation of Conditional Admission (VT), and supports the Cabinet's Vision on Biotechnology 2025-2040. Together, these mandates underline the urgency of adopting a structured and sustainable solution.

Calls to Action and Next Steps

- **October 2025**: Stress-test the framework at the Roundtable with policymakers, clinicians, pharmacists, and patients.
- **2026:** Launch a co-designed pilot (patients + clinicians lead; insurers support)
- **2026/27**: Use pilot results to inform a national adoption decision by VWS and ZIN in a future proof system.
- **2027 onward**: Share outcomes into the EU Access Toolbox and HTA 2028 debate, positioning NL as a frontrunner

> *"Fast Track does not lower standards; it designs equity, evidence, and accountability into the system from the start."*

Fast Track – Key Questions

Patients

1. ***How does Fast Track ensure outcomes reflect what matters to patients?***
   Fast Track embeds patient expertise as infrastructure. Patients co-define outcomes, organisations shape care pathways, and patient leaders participate directly in governance.
2. ***Will this create unequal access for rare disease patients compared to others?***
   No. Fast Track supplements existing procedures: it does not replace them. It ensures proportionate, transparent availability of treatments for small populations where the current system struggles.

Clinicians & Pharmacists

3. ***What role do clinicians and pharmacists play?***
   They co-design and lead treatment protocols, collect real-world evidence, and ensure feasibility in daily practice.
4. ***Does this increase administrative burden?***
   The framework streamlines existing fragmented pathways (WGA, ODAP, VT, Sluis) into a proportionate and efficient route. Pilots will test and refine to avoid unnecessary duplication.

Insurers

5. ***How is affordability safeguarded?***
   Insurers are engaged as observers in the pilot to ensure sustainability. Decisions are tiered and based on confidence, so risk is addressed, and costs are proportionate to evidence and need.
6. ***How does Fast Track deal with cost-effectiveness in rare diseases?***
   Traditional cost-effectiveness analysis is often inappropriate for rare and ultra-rare diseases, because very small patient numbers create variability and ICERs that do not reflect real value. Instead, Fast Track applies proportionate criteria that factor in rarity, unmet need, and patient relevance while still safeguarding affordability and quality of care.

Policymakers

7. ***How does this link to Fleur Agema's Cabinet letter and the Paulusma/Bushoff motion?***
   Fast Track operationalises both the Cabinet letter from the then-Minister of Health Fleur Agema (2024), which committed to developing a fast track procedure for a select group of medicines so that patients with high unmet need do not have to wait unnecessarily for clarity on reimbursement[8], and the Paulusma/Bushoff motion[2] (2024), which called for faster access routes for orphan drugs. Fast Track provides a concrete, patient-first model to deliver on these commitments in practice and is designed to be implementable under the current legal framework.

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[8] Motion by members Paulusma and Bushoff. (2024, June 12). Tweede Kamer der Staten-Generaal. https://www.tweedekamer.nl/kamerstukken/moties/detail?id=2024Z10302&did=2024D24327. Accessed July 2025

[2] Minister of Health Fleur Agema, Kamerbrief over geneesmiddelenbeleid (4 October 2024).
"Ten eerste, het inrichten van een versnelde procedure voor een selecte groep geneesmiddelen waardoor patiënten in hoge nood minder lang hoeven te wachten op duidelijkheid over vergoeding van een nieuw middel – een zogeheten 'fast track'…"

8. ***Is this compatible with EU frameworks?***
   Yes. It positions the Netherlands as a frontrunner nationally first, while contributing to the EU Access Toolbox and aligning with the HTA Regulation ahead of 2028.
9. ***Does Fast Track replace current procedures?***
   No. It is a supplementary route, designed to complement and streamline existing and future pathways. No legislative change is required to test the pilot.
10. ***How does the Netherlands compare to other EU countries on availability of treatments for patients with rare disease?***
    The Netherlands is already falling behind. Patients here often wait years for therapies that are available much sooner in neighbouring countries. Fast Track offers a sustainable, patient-first pathway to close this gap and ensure Netherlands keeps pace with European ambitions for earlier and more equitable availability of treatments for patients with rare disease.

System

11. ***How will success be measured?***
    Through a co-designed pilot in 2026, evaluating feasibility, patient relevance, commitment and sustainability. Results will inform a national adoption decision and feed into the EU debate.
12. ***What is the timeline?***
    October 2025: Roundtable stress-test.
    2026: Pilot design and implementation.
    Q4 2026/early 2027: National adoption decision.

Annex 1: Detailed Assessment Criteria

The Fast Track framework applies six criteria to determine the confidence tier for treatments in rare and ultra-rare diseases where uncertainty is high. These criteria reflect both patient priorities and system needs.

Criterion	High Confidence	Moderate Confidence	Low Confidence/DEVELOP
Patient Relevance	Patients co-designed research: outcomes reflect lived priorities; equal partnership in decisions	Patient perspective acknowledged; some community dialogue; partial integration	Limited patient input: outcomes defined primarily by clinical/payer perspectives
Necessity	High patient-reported burden; no effective alternatives; clear unmet need	Moderate burden; some alternatives; mixed patient priorities	Lower burden; adequate alternatives available
Effectiveness	Strong evidence for patient-relevant outcomes and appropriate use; high confidence in real-world benefit	Moderate confidence: outcomes partially align with patient priorities	Limited confidence in meaningful patient benefit
Cost-Effectiveness	Acceptable when considering patient-defined value and rarity context	Uncertain but includes patient-relevant considerations	Challenging even with patient priorities considered
Feasibility	Can be delivered equitably to all eligible Dutch patients; infrastructure ready	Minor implementation gaps: some availability considerations needed	Implementation challenges requiring significant development if expert centers are not ready
Budget Impact	Proportionate to patient population and need	Moderate impact requiring collaborative management	Substantial impact requiring extensive coordination

Annex 2: Glossary

Conditional Admission (VT)
Dutch system allowing temporary reimbursement of promising therapies under strict conditions, typically for up to seven years, with evaluation based on additional evidence collected during that period.

Empowered By Us (EBU)
Patient-led initiative focused on inclusion, equity, and rare disease policy in the Netherlands and Europe. Co-author of the Fast Track framework.

European Medicines Agency (EMA)

EU agency responsible for scientific evaluation, supervision, and safety monitoring of medicines. EMA approval is a prerequisite for market entry but does not guarantee reimbursement at national level.

EU Access Toolbox (2025)
European initiative to support earlier and more equitable availability of orphan and innovative therapies. Calls for pilots, outcome-based models, and patient involvement.

Fast Track NL
Proposed supplementary pathway to provide earlier, proportionate availability of treatments for rare and ultra-rare diseases where uncertainty is high, using a confidence-based tiering system.
Note: In the Dutch regulatory version, these tiers are expressed as High / Moderate / Low (Develop) to align with terminology used by VWS and ZIN. The English version uses GO / CONDITIONAL / REVIEW for accessibility and policy communication purposes.

GRADE
Grading of Recommendations, Assessment, Development and Evaluations. Common evidence assessment method, often poorly suited to very small, rare disease populations.

Insurer (Zorgverzekeraars)
Private health insurance companies operating within the Dutch system who cover patient treatments. Responsible for implementing reimbursement decisions made by VWS and ZIN.

National Plan Rare Diseases (NPRD)
Dutch government plan (under development) intended to strengthen rare disease care and policy. Provides broader context but is separate from Fast Track.

Orphan Drug (OD)
Medicine intended for diagnosis, prevention, or treatment of a life-threatening or chronically debilitating rare disease or condition affecting fewer than 5 in 10,000 people in the EU.

Orphan Drug Arrangement (WGA)
Dutch pathway for managed entry of orphan medicines, based on negotiated agreements on use and monitoring.

ODAP (Orphan Drug Access Protocol)
Pilot protocol used in the Netherlands for collecting structured data on orphan drug use.

Paulusma/Bushoff Motion (2024)
Parliamentary motion mandating faster access route for orphan drugs. Remains an unmet political commitment that Fast Track operationalises.

Sluis (Lock System)
Dutch mechanism requiring medicines with projected annual costs above €10M to undergo additional evaluation before reimbursement.

VWS (Ministry of Health, Welfare and Sport)
Dutch ministry responsible for healthcare policy, including reimbursement and pricing decisions.

ZIN (Zorginstituut Nederland / National Health Care Institute)
Government agency advising the Dutch government on coverage decisions for medicines and treatments.