ALAN

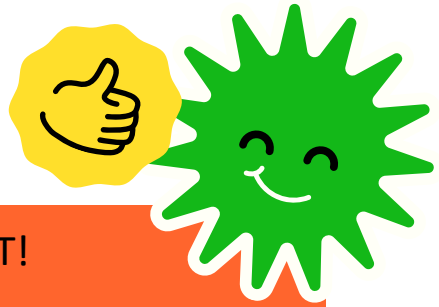
Acute Leukemia Advocates Network

GLOBAL SUMMIT
7TH – 9TH NOVEMBER 2025

www.acuteleuk.org

STAYING ENGAGED & EXPANDING IMPACT

TODAY'S AGENDA



8.15 AM	Breathing / Mindfulness
8.30 AM	Access – Global Realities and Solutions
9.30 AM	Power in Partnership
10.30 AM	Buddy Check in
11 AM	Workshop: What to Advocate for Locally?
11.45 AM	Closing and goodbye
12.30 PM	Lunch and departures

DON'T FORGET!

- We do not expect anyone to be an observer - Everyone is welcome and encourage to participate
- Activities proposed during the breaks are optional
- Bad english is the official language

TO-DOS

- Be on time !
- Keep your badge on
- Mute your phone and switch off your computer

GLOBAL REALITIES AND SOLUTIONS


Solution
Problem

Understanding the differences in policies and environments across countries and strategies to overcome them

- Patient participation in the Joint Clinical Assessments (European Health Technology Assessments)
- Shaping Policy to Deliver Modern Therapies in New Zealand
- Access to Acute Leukemia Care: What is it like in LMICs ?

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PATIENT PARTICIPATION IN JOINT CLINICAL ASSESSMENTS

Anne-Pierre Pickaert – November 9, 2025

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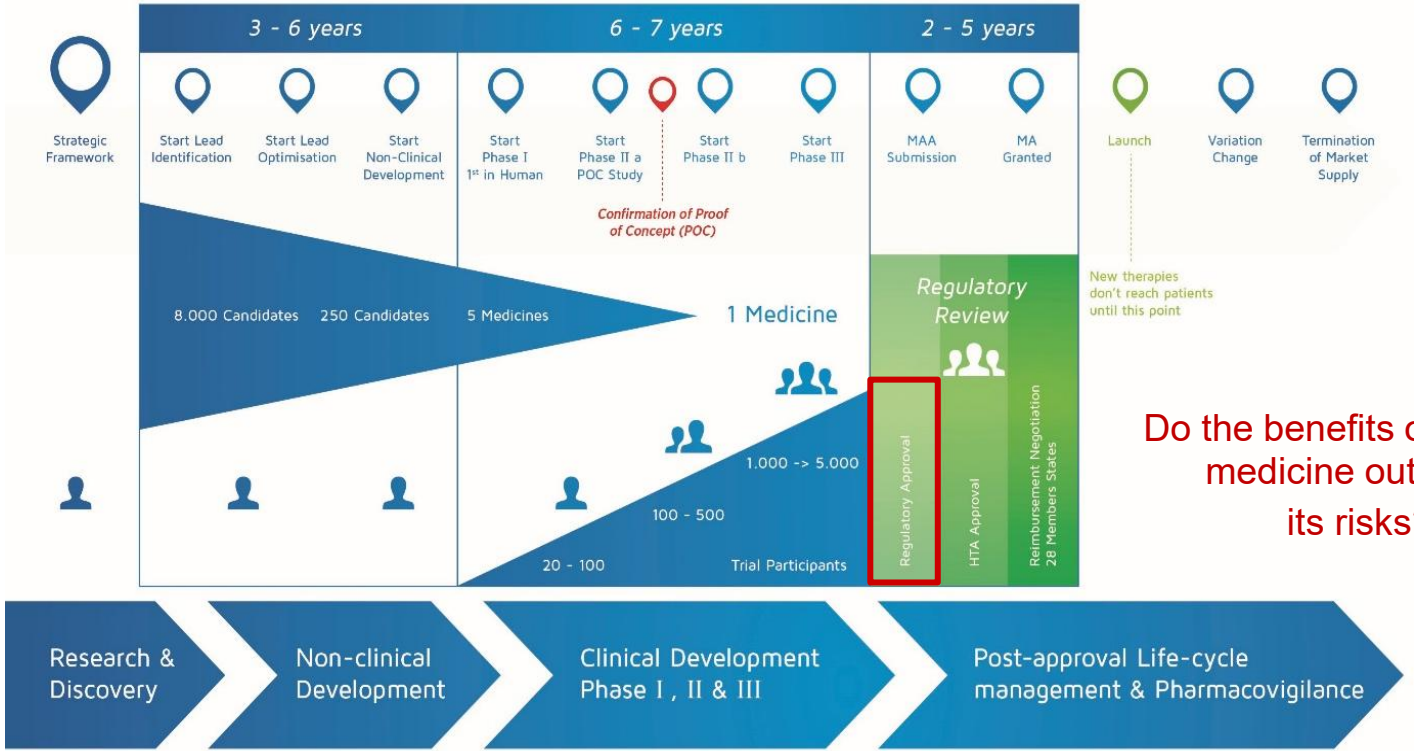
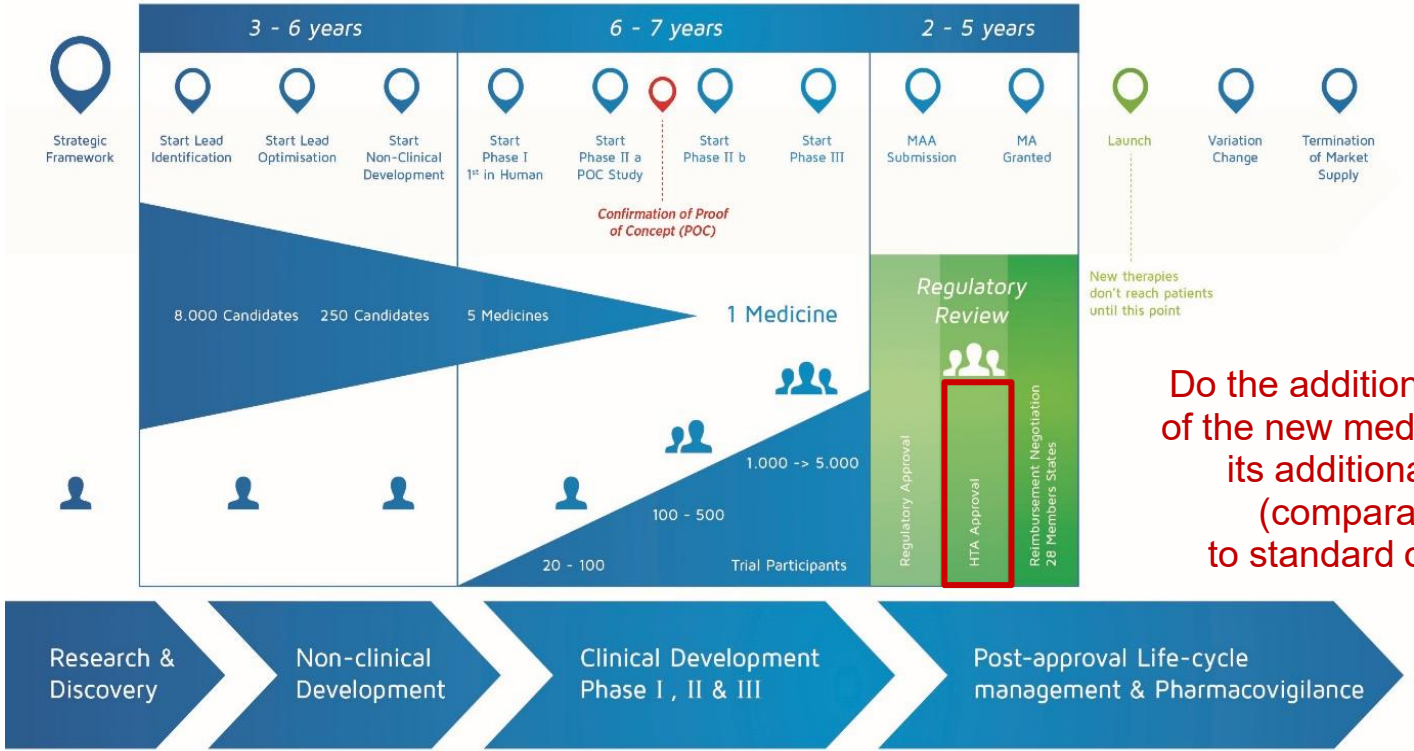
Marketing authorisation process focuses on absolute benefits



Do the benefits of the new medicine outweigh its risks?

https://toolbox.eupati.eu/resources/marketing-authorisation-applications/

Health Technology Assessment (HTA) focuses on relative benefits



Do the additional benefits of the new medicine justify its additional costs (comparatively to standard of care)?

https://toolbox.eupati.eu/resources/marketing-authorisation-applications/

How is this new medicine doing comparatively to existing treatments?

HTA looks at

- How the medicine works
- Whether it is safe
- Whether the benefits it offers is worth the cost.

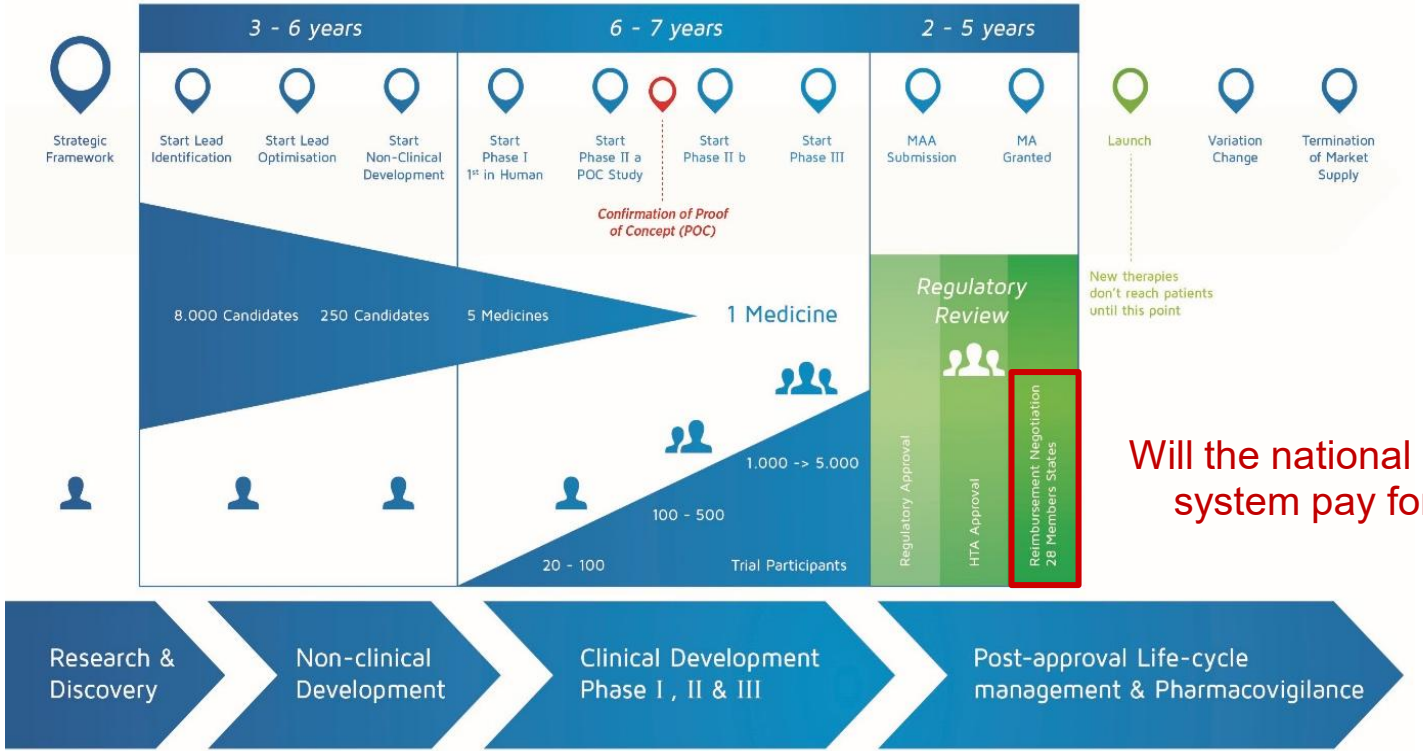
HTA has two main parts

- **Assessment**: This is the scientific review.

- **Appraisal**: This is where the evidence is interpreted, and coverage recommendations are given

Le Chat Mistral

Reimbursement determines if & how patients can access the medicine



Will the national health system pay for it?

https://toolbox.eupati.eu/resources/marketing-authorisation-applications/

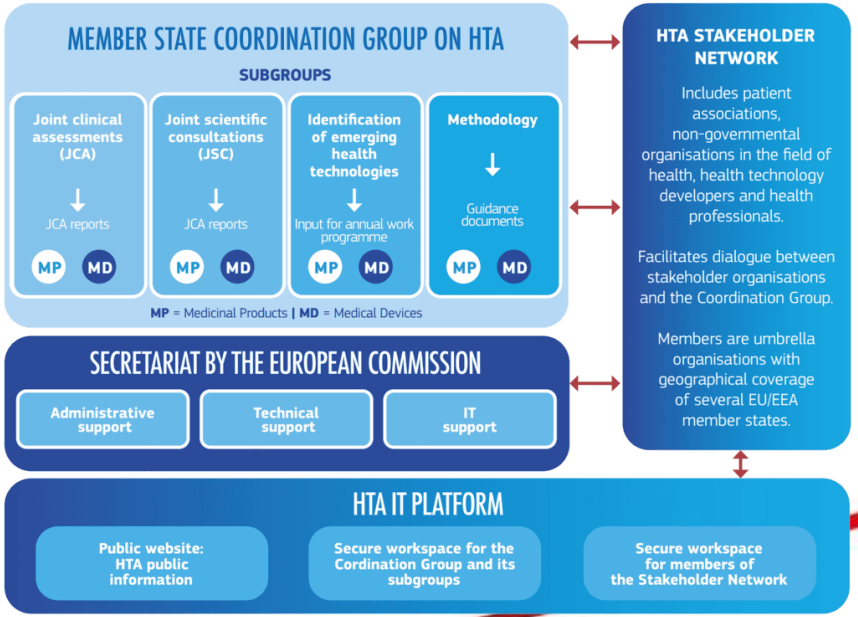
What is new since 2025? A European Joint HTA

European countries now work more closely together on evaluating how new medicines are evaluated comparatively to existing treatments

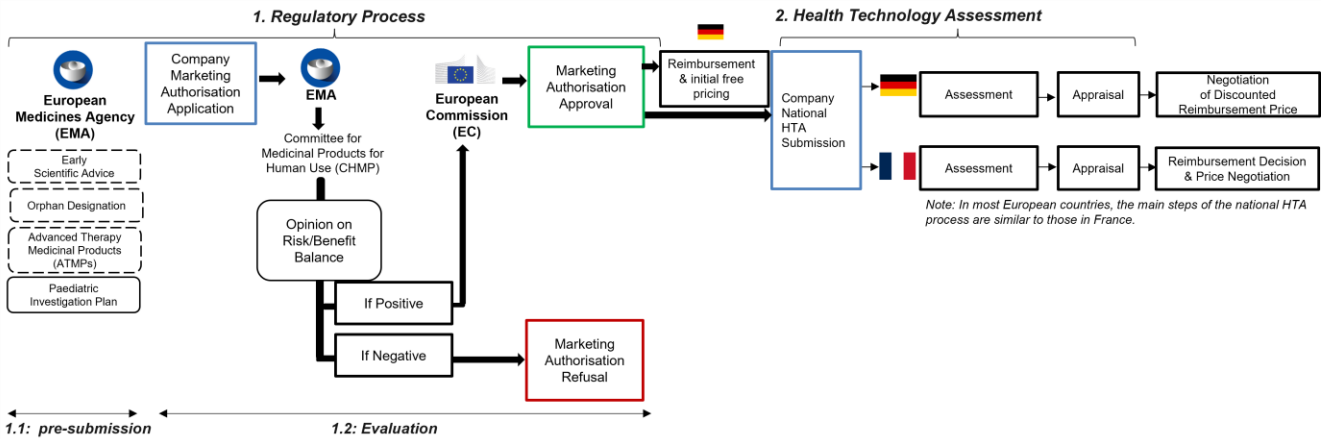
Objectives

- Reduce duplication of effort across Member States
- Enhance transparency and scientific quality
- Support timely and equitable access to innovative technologies.
- Facilitate involvement of key stakeholders, including patients

Governance



Marketing authorization and HTA processes until 2024

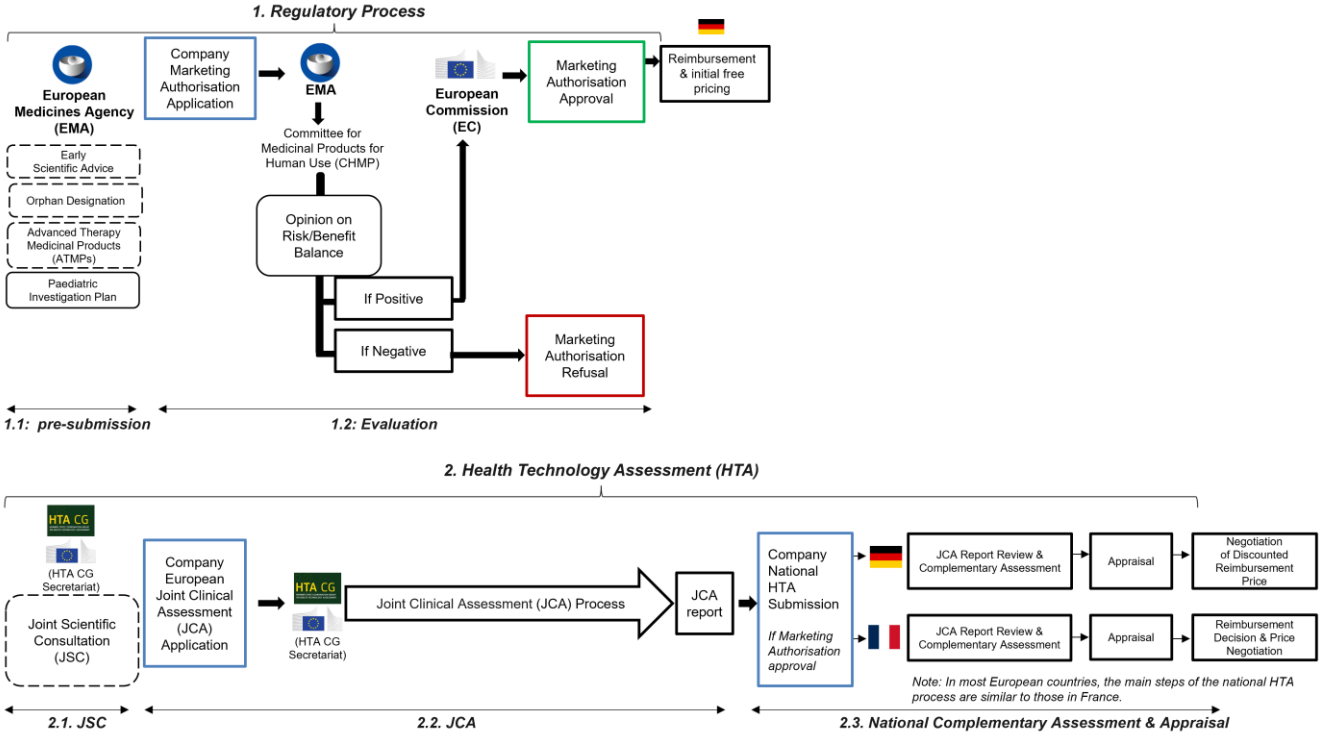


Not legally required

Chart adapted from Haute Autorité de Santé (HAS).

Acute Leukemia Advocates Network (ALAN).. How Medicines Are Evaluated in Europe https://acuteleuk.org/drug-evaluation/

Marketing authorization and HTA processes since 2025



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Acute Leukemia Advocates Network (ALAN).. How Medicines Are Evaluated in Europe https://acuteleuk.org/drug-evaluation/

Ongoing JCAs: new molecules in oncology & ATMPs

Treatment of melanoma
Assessor: HAS, **France**
Co-assessor: AOTMiT, **Poland**

Treatment of paediatric low-grade glioma
Assessor: NCPE, **Ireland**
Co-assessor: IQWIG, **Germany**

Treatment of bladder cancer
Assessor: ZIN, **Netherlands**
Co-assessor: DMC, **Denmark**

Treatment of 5q spinal muscular atrophy
Assessor: NCPE, **Ireland**
Co-assessor: HAS, **France**

Maintenance treatment of extensive-stage small cell lung cancer
Assessor: IQWIG, **Germany**
Co-assessor: INFARMED, **Portugal**

Treatment of locally advanced or metastatic breast cancer
Assessor: DVSV, **Austria**
Co-assessor: RIZIV- INAMI, **Belgium**

Treatment of extensive-stage small cell lung cancer
Assessor: IQWIG, **Germany**
Co-assessor: NCPHP, **Hungary**

Treatment of synovial sarcoma or leiomyosarcoma
Assessor: TLV, **Sweden**
Co-assessor: NOMA, **Norway**

Maintenance treatment of advanced epithelial high-grade ovarian, fallopian tube or primary peritoneal cancer
Assessor: IQWIG, **Germany**
Co-assessor: Slovenian Quality Health Care Agency, **Slovenia**

Treatment of adult patients with platinum-resistant epithelial ovarian, fallopian tube, or primary peritoneal cancer in combination with nab-paclitaxel
Assessor: INFARMED, **Portugal**
Co-assessor: SBU, **Sweden**

Engagement of European patients in joint clinical assessments

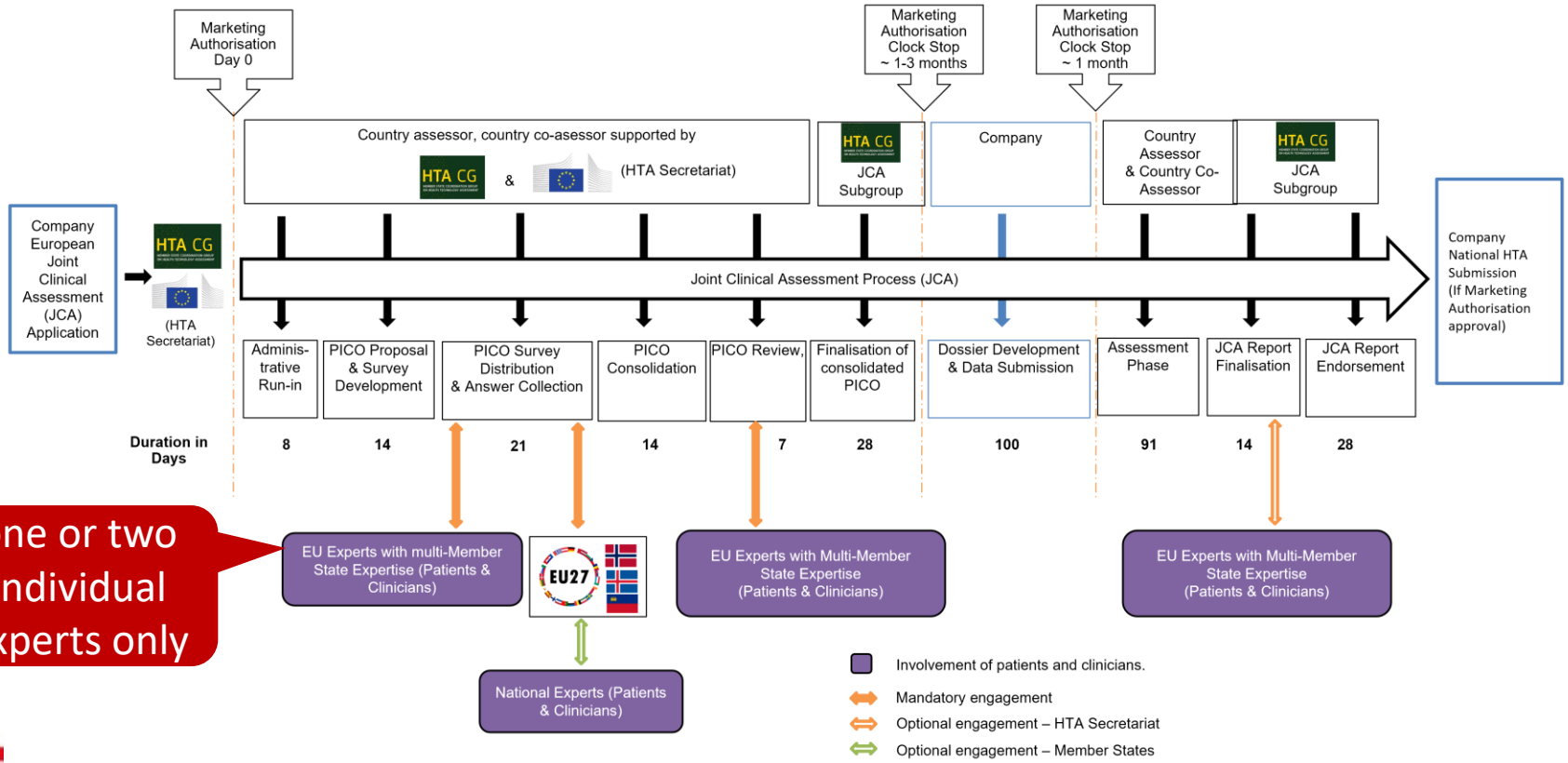

Chart adapted from Haute Autorité de Santé (HAS).
Acute Leukemia Advocates Network (ALAN).. How Medicines Are Evaluated in Europe https://acuteleuk.org/drug-evaluation/

Patient selection and conflict of interest

EC request: a certain number of potential experts identified (3 weeks), contact details sent to EC

Before any conversation with someone, by phone or email, they receive a link to something like this

After others gave up, 5 register and fill-in forms

3 proposed to the JCA subgroup

Of whom 2 are selected. Criteria? Explanation provided?

EC assesses CoI, not the profiles

What if these 2 have better profiles, eg involved in treatment guidelines? Why not balance expertise and conflict Why not let the JCA subgroup decide who to involve among all 5?

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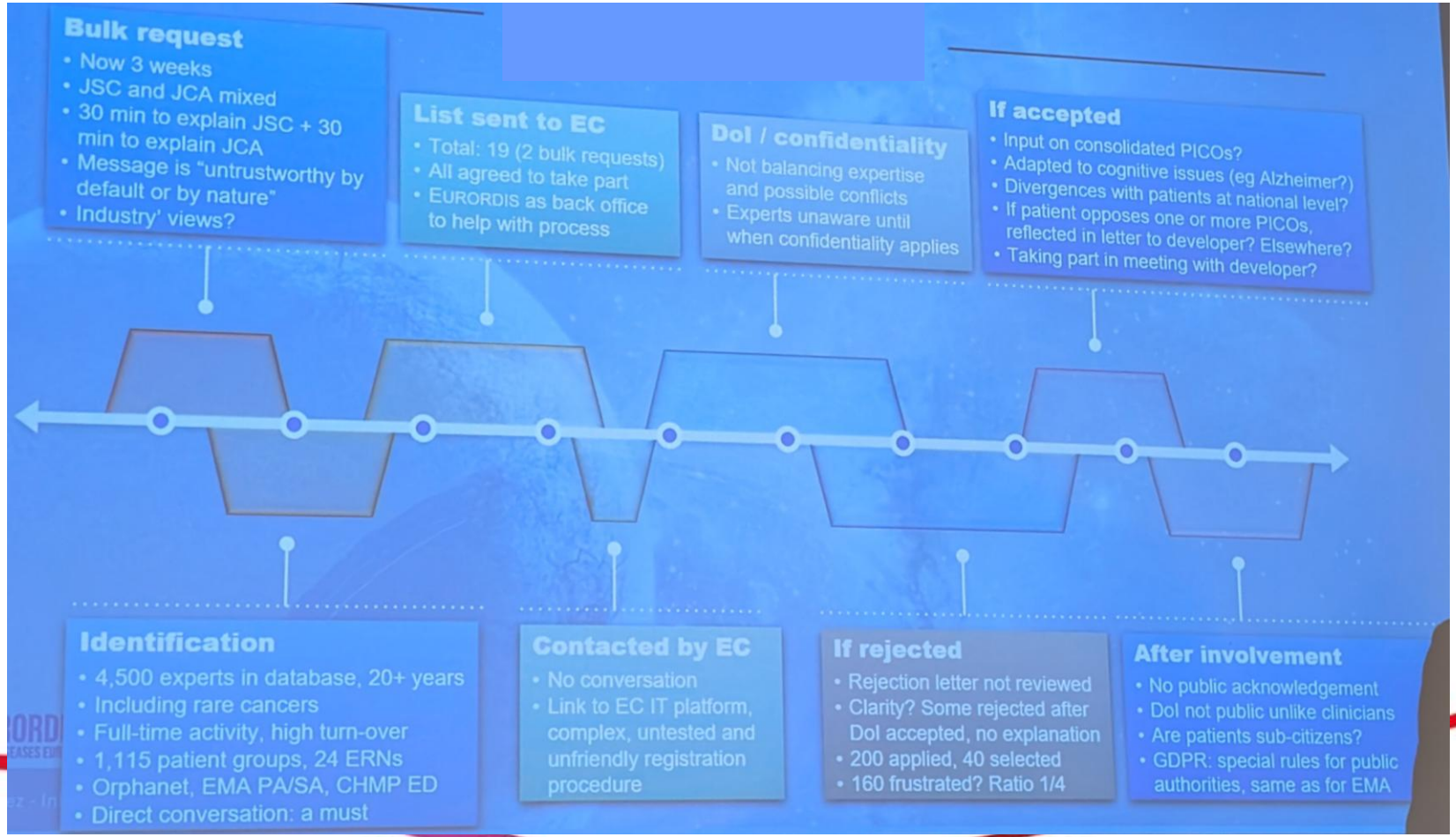
RARE DISEASES EUROPE

F. Houÿez - Information and Access Director | EURORDIS

How it is done & questions



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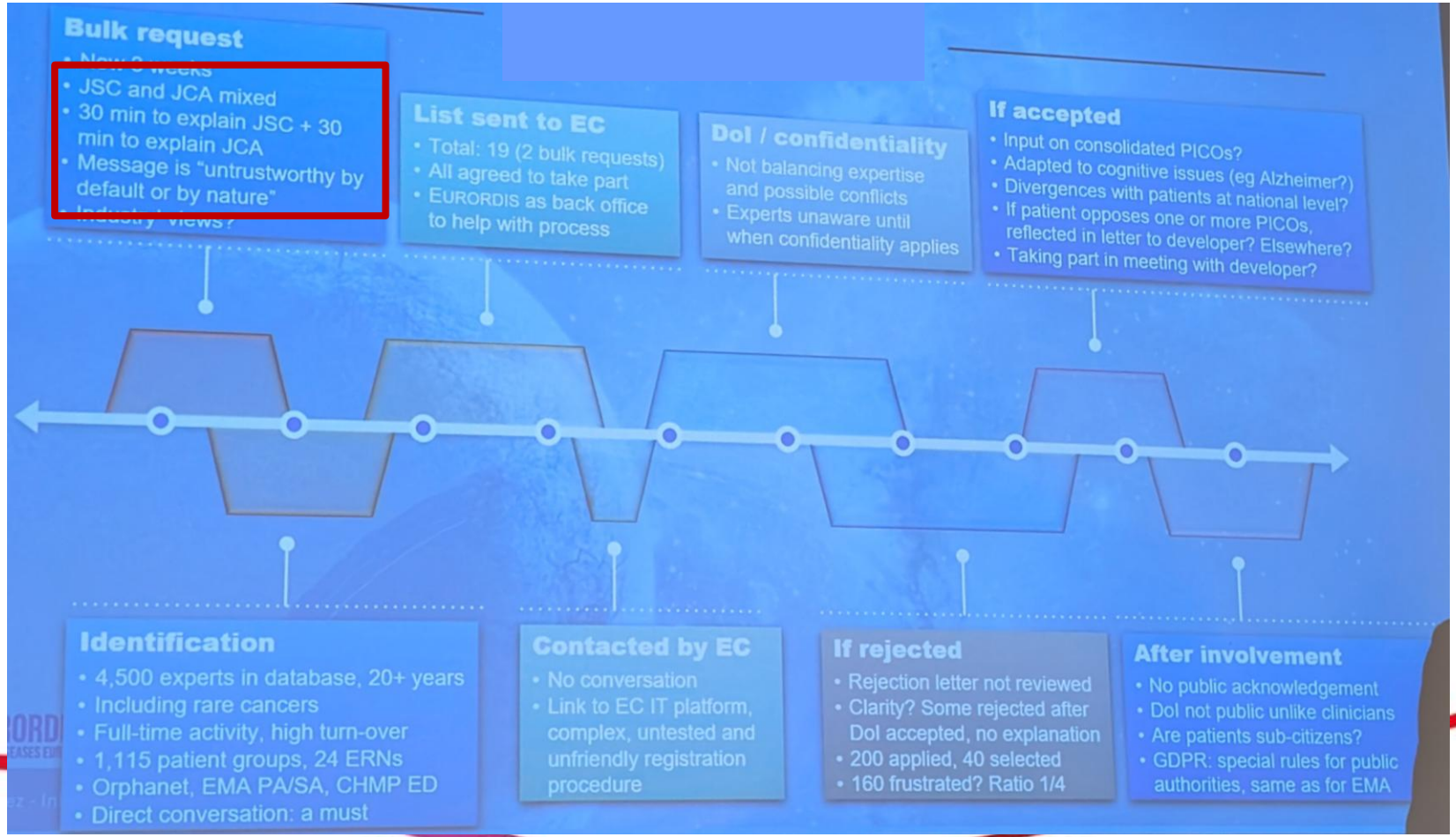

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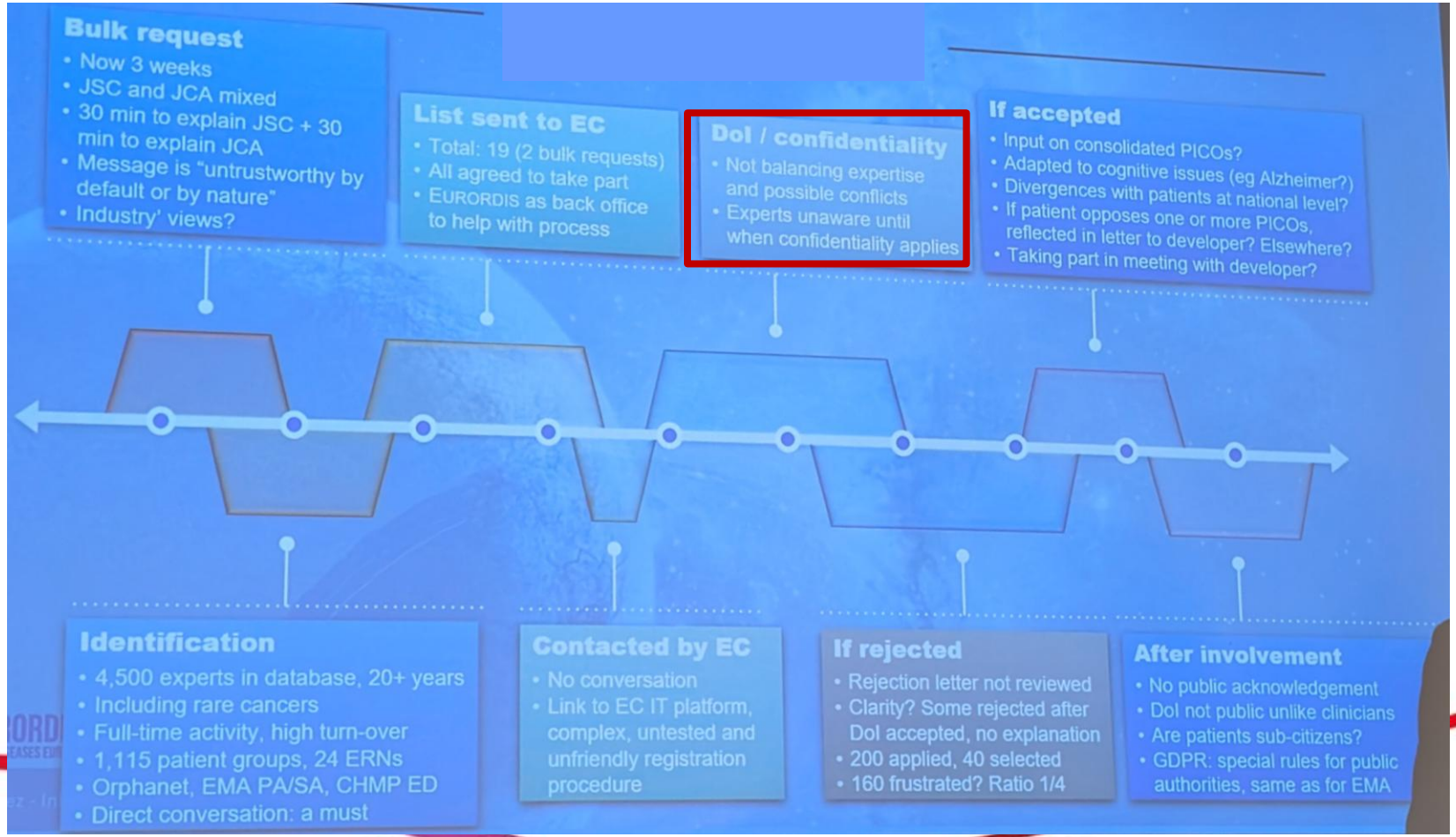
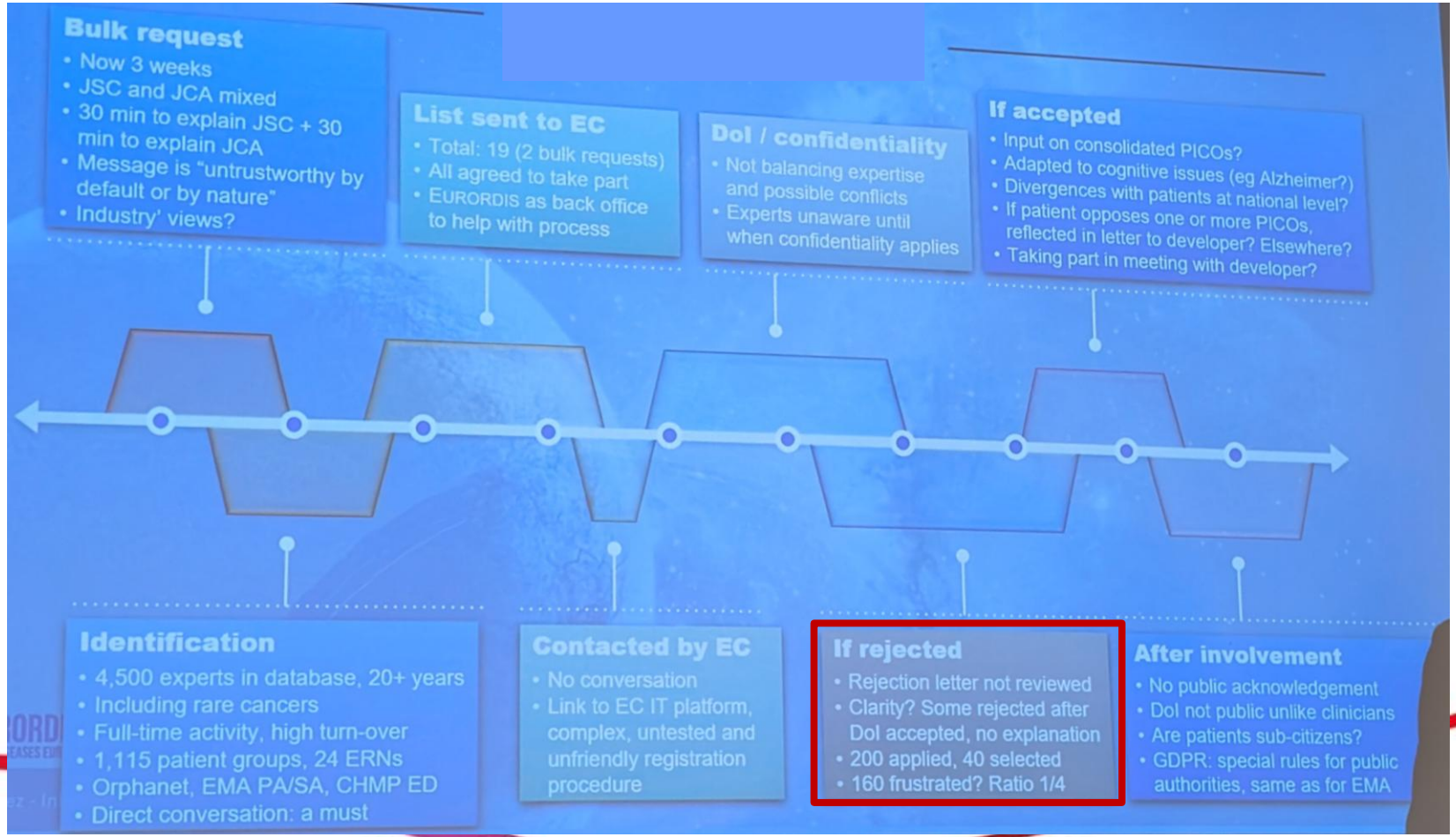
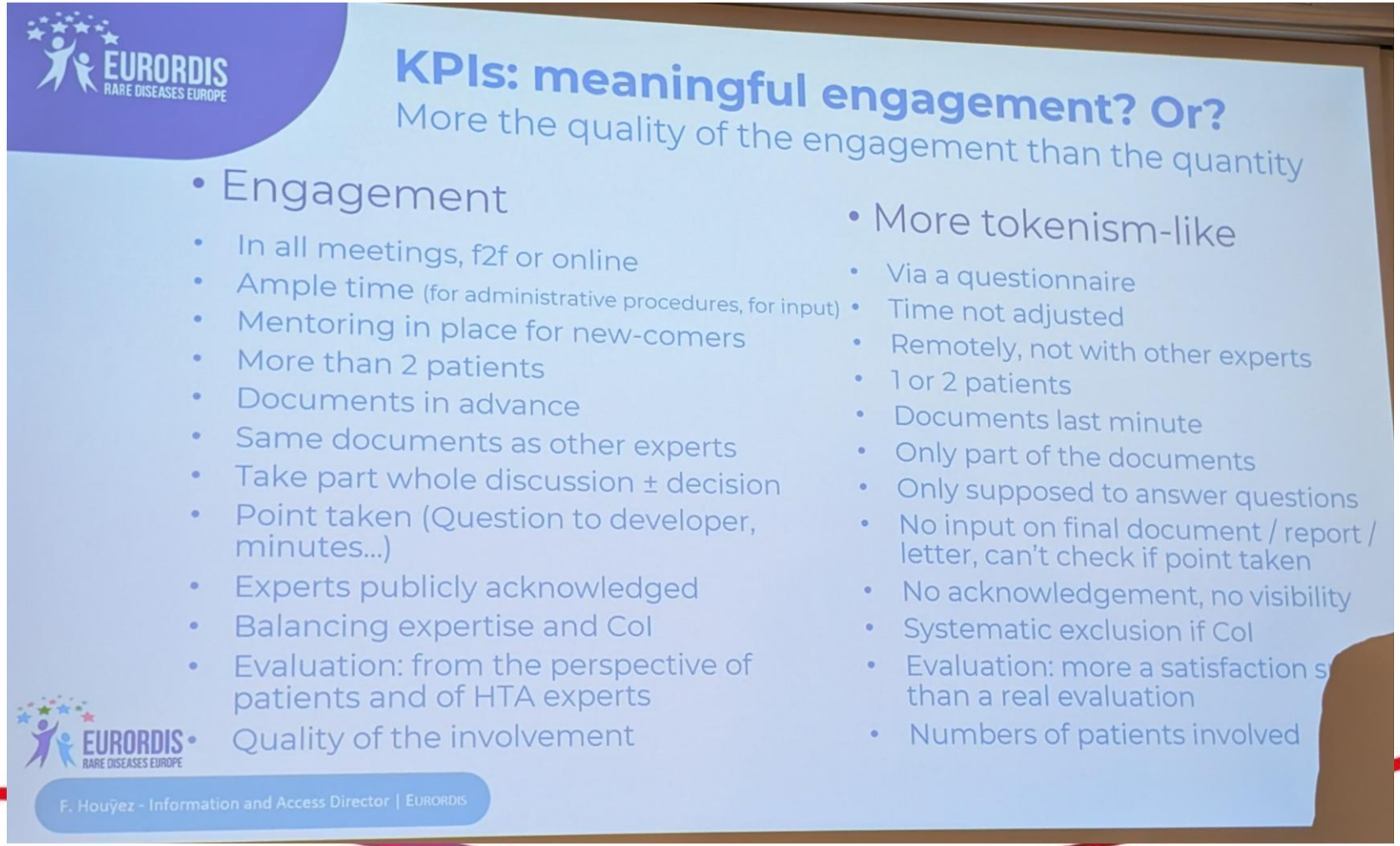


How it is done & questions

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KPIs: meaningful engagement? Or?
More the quality of the engagement than the quantity
• Engagement
• In all meetings, f2f or online
• Ample time (for administrative procedures, for input)
• Mentoring in place for new-comers
• More than 2 patients
• Documents in advance
• Same documents as other experts
• Take part whole discussion ± decision
• Point taken (Question to developer, minutes…)
• Experts publicly acknowledged
• Balancing expertise and CoI
• Evaluation: from the perspective of patients and of HTA experts
• Quality of the involvement
• More tokenism-like
• Via a questionnaire
• Time not adjusted
• Remotely, not with other experts
• 1 or 2 patients
• Documents last minute
• Only part of the documents
• Only supposed to answer questions
• No input on final document / report / letter, can't check if point taken
• No acknowledgement, no visibility
• Systematic exclusion if CoI
• Evaluation: more a satisfaction s
than a real evaluation
• Numbers of patients involved
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THANK YOU!

Shaping Policy to Deliver Modern Therapies in New Zealand

Rosie Shaw, Head of Advocacy

Leukaemia & Blood Cancer NZ

leukaemia & blood cancer NEW ZEALAND
Vision to Cure. Mission to Care.

Our Impact

1 Patient Support

Deliver valued patient and family support services nationally

2 Research

Proactively drive research into the prevention, cure and care of blood cancer in New Zealand

3 Advocacy

Advocate for access to the best global standard of treatment for New Zealand patients

Fundraising & Sustainability

Turning generosity from New Zealanders into improved outcomes for patients

Improving care in New Zealand through patient-centred advocacy

ALLOGENEIC STEM CELL TRANSPLANT ACCESS

MEDICINE ACCESS & CAR T-CELL THERAPY

STATE OF THE NATION & FUTURE ROADMAP

Stem cell transplant access in New Zealand

HEALTH & SCIENCE

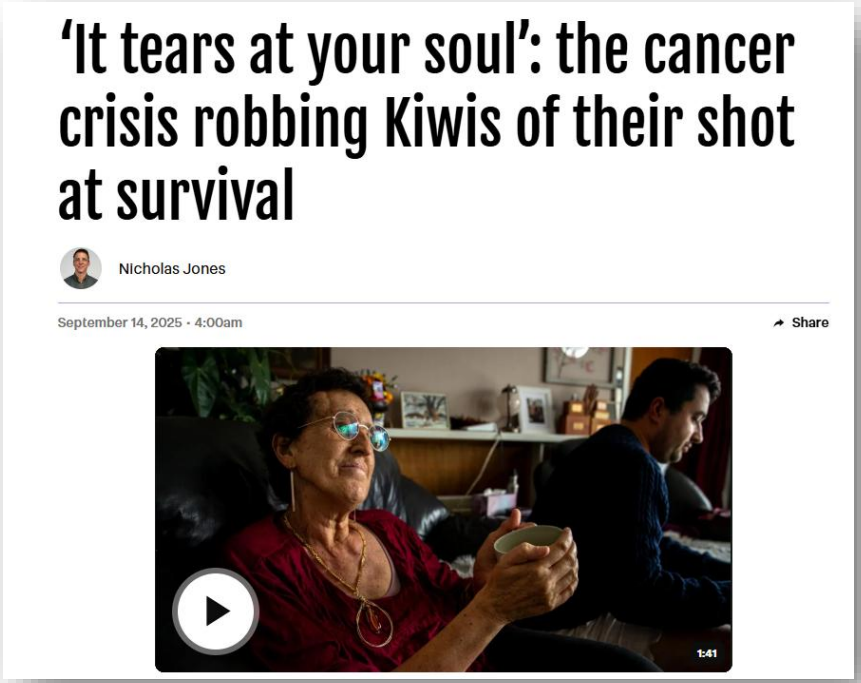
Cancer patient may die after months languishing on waitlist

New Zealand is one of the few countries that runs a waitlist for stem cell transplants, costing patients their lives and the health system money

NEW ZEALAND / HEALTH

Cancer patient waits six months for bone marrow transplant

Stem cell transplant access in New Zealand



‘It tears at your soul’: the cancer crisis robbing Kiwis of their shot at survival

Nicholas Jones

September 14, 2025 · 4:00am

Share

1:41

Stem cell transplant access in New Zealand

Health New Zealand
Te Whatu Ora

Achieving the Health Targets

High Level Implementation Plans

July 2024 – June 2027

Faster cancer treatment

2030 Health Target

90% of patients to receive cancer management within 31 days of the decision to treat

2024-27 GPS performance milestones

2024/25	2025/26	2026/27
86%	87%	88%

Initiatives	July 2024–June 2027 Y1	Y2	Y3
Action 1. Address unwarranted variation in access to care			
Develop **national standards and operating model** to address unwarranted variation across the country. • Increased capacity for allogeneic stem cell transplantation at Auckland, Wellington and Christchurch. • Create regional integrated radiation oncology services to meet demand and drive down variation in intervention rates. • Phased approach to wider distribution of ambulatory chemotherapy to local sites.	✓	✓	✓

Health New Zealand
Te Whatu Ora

Achieving the Health Targets

Faster cancer treatment

2030 Health Target	2024–27 GPS performance milestones

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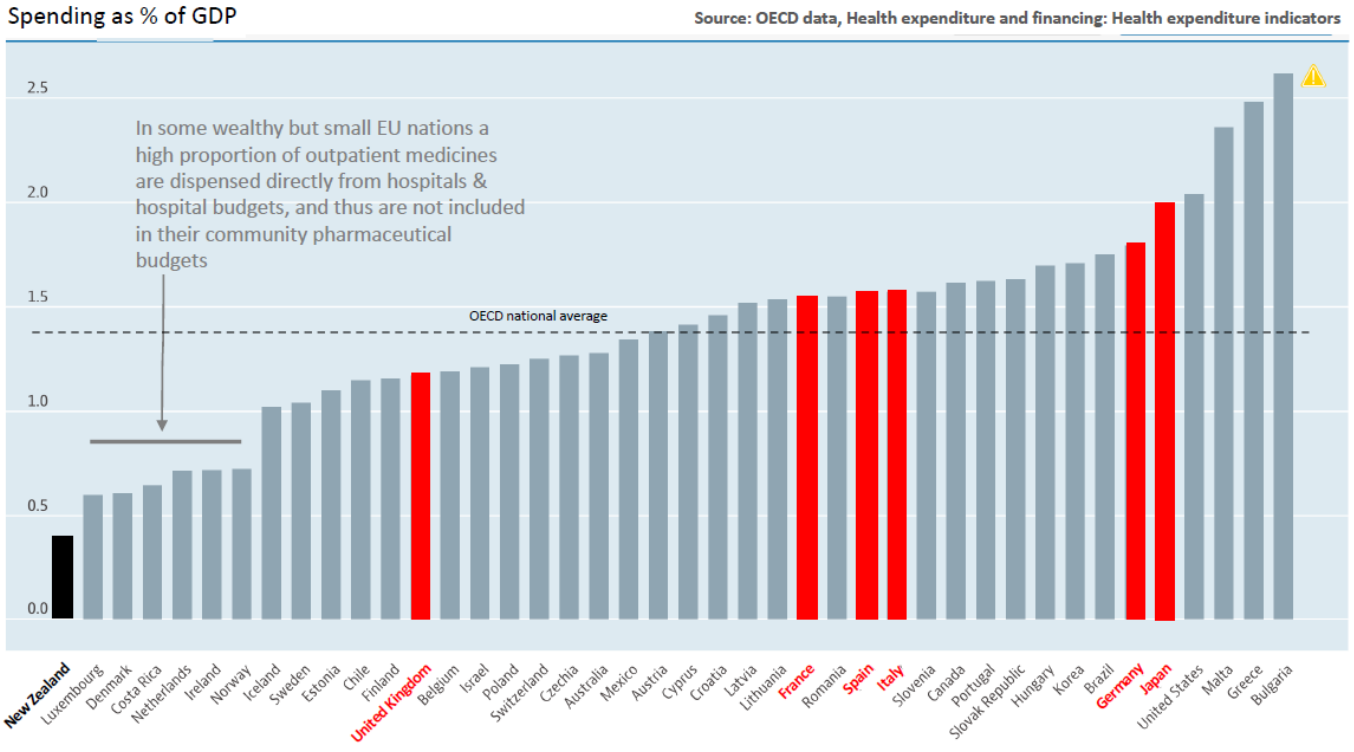
- Create regional integrated radiation oncology services to meet demand and drive down variation in intervention rates.
- Phased approach to wider distribution of ambulatory chemotherapy to local sites.

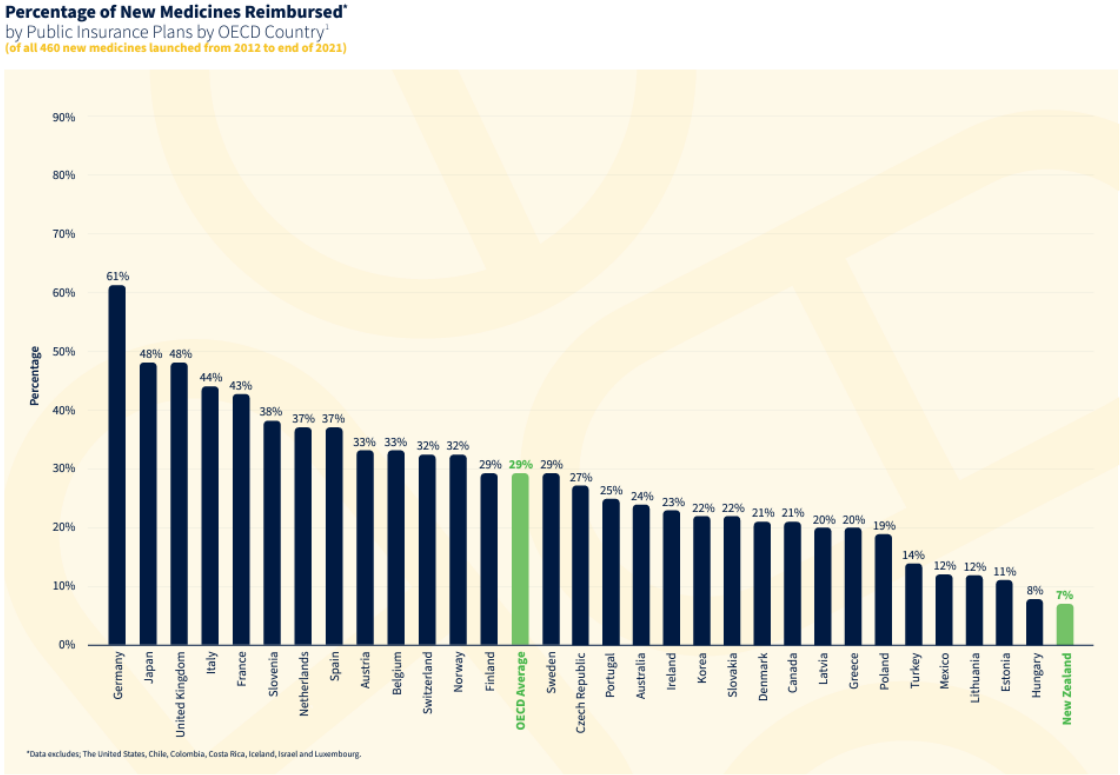
leukaemia & blood cancer NEW ZEALAND

Medicine access in New Zealand



https://data.oecd.org/healthres/pharmaceutical-spending.htm; https://www.nzier.org.nz/; https://www.insights10.com/report/new-zealand-over-the-counter-otc-pharmaceuticals-market-analysis/

Medicine access in New Zealand

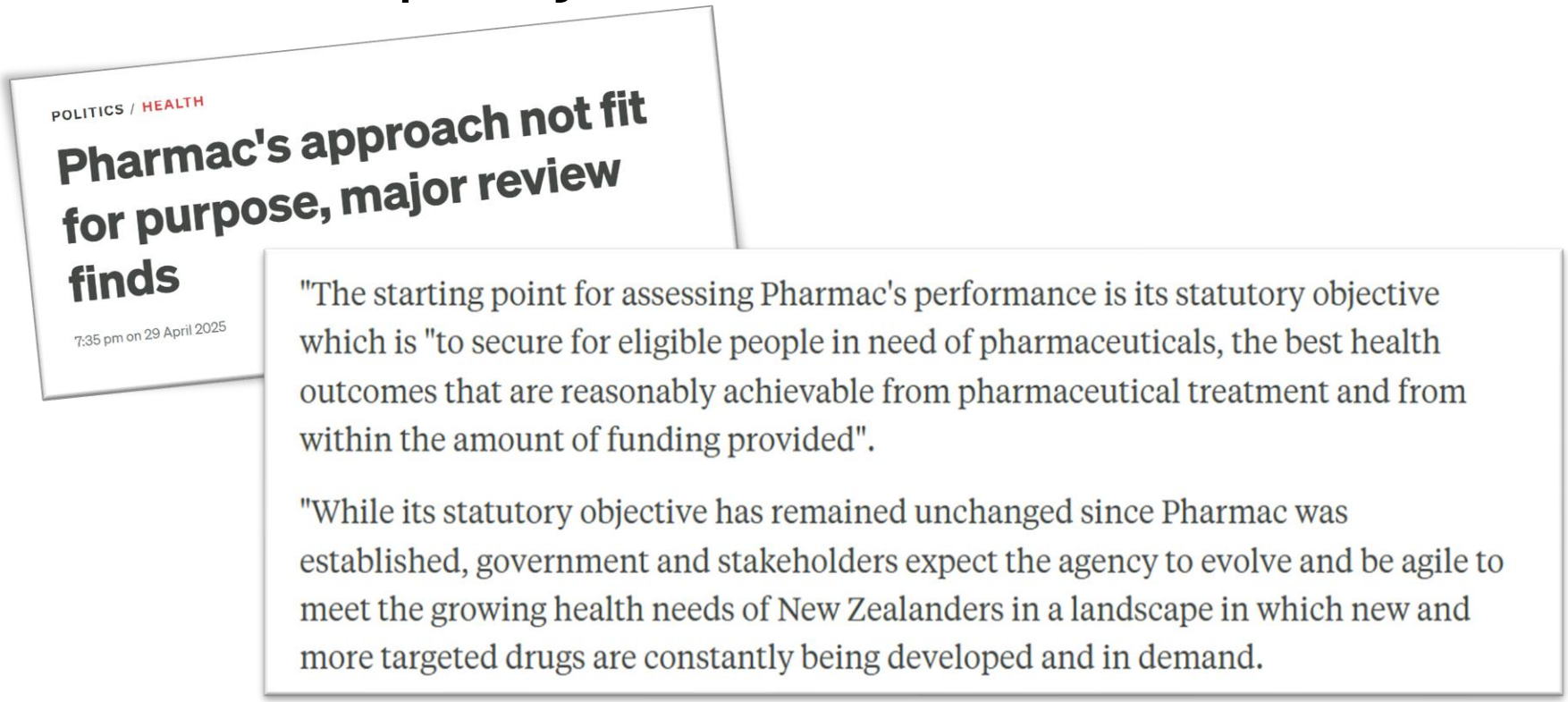


https://www.medicinesnz.co.nz/fileadmin/assets/MED_5355_Landscape_2024_FA_WEB1__1_.pdf

The medicine gap for acute leukaemia

Key NCCN-recommended medicines not funded in NZ	
AML	• **Quizartinib** – FLT3-ITD-positive AML (first-line) • **Gilteritinib** – Relapsed/refractory FLT3-mutated AML • **Ivosidenib** – IDH1-mutated AML • **Enasidenib** – IDH2-mutated AML • **CPX-351** (daunorubicin/cytarabine liposome) – Secondary AML or AML with myelodysplasia-related changes • **Oral azacitidine** (Onureg) – Post-remission maintenance therapy
ALL	• **Blinatumomab** – B-cell ALL (MRD-positive or relapsed/refractory) • **Nelarabine** – T-cell ALL (relapsed/refractory) • **Ponatinib** – Philadelphia-positive ALL (resistant or intolerant to other TKIs) • **CAR T-cell therapies** – Kymriah and Tecartus

Medicine policy in New Zealand



POLITICS / HEALTH

Pharmac's approach not fit for purpose, major review finds

7:35 pm on 29 April 2025

"The starting point for assessing Pharmac's performance is its statutory objective which is "to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided".

"While its statutory objective has remained unchanged since Pharmac was established, government and stakeholders expect the agency to evolve and be agile to meet the growing health needs of New Zealanders in a landscape in which new and more targeted drugs are constantly being developed and in demand.

Policy shaping for system-level change

Raised the issue directly with Parliament through the Health Select Committee

Represented on Pharmac's Consumer & Patient Working Group, informing the Reset Programme

Collaborating with 10 Cancer NGOs to inform medicine policy reform ahead of the next election

MAKE MEDICINE ACCESS NON NEGOTIABLE

A unifying platform built for adoption.

Clear actions that build pressure and secure change.

On pace for the election, made with purpose.

Doyle
Dane
Bernbach

State of the Nation & Future Roadmap

UNIVERSITY OF AUCKLAND
Waipapa Taumata Rau
NEW ZEALAND

Deloitte.

TE AHO O TE KAHU
CANCER CONTROL AGENCY


Vision: No lives needlessly lost to blood cancer by 2035
rosie.shaw@leukaemia.org.nz

ACCESS TO ACUTE LEUKEMIA CARE: WHAT IS IT LIKE IN LMICS?

ELO MAPELU

HENZO KENYA AND THE MAX FOUNDATION

- Acute Leukemia (AL) is an emergency. The difference between life and death often comes down to *where* you are born.

- **The Divide (HICs vs. LMICs):**

  High-Income Countries (HICs): 5-year survival for ALL is about 80% Driven by advanced diagnostics, targeted therapies, and robust supportive care.

  Low- and Middle-Income Countries (LMICs): 5-year survival for ALL is about 20%

  The Gap is a Policy Failure.

UNDERSTANDING THE POLICY & ENVIRONMENTAL DIFFERENCES

Factor	High-Income Countries (HICs)	Low/Middle-Income Countries (LMICs)
Diagnostics	Standard: Flow Cytometry, Karyotyping, NGS (Molecular Profiling).	Challenge: Rely on basic CBC. Advanced tests (e.g., FLT3, NPM1 for AML) are scarce, costly, or absent.
Treatment Access	Wide availability of standard and novel agents (e.g., Venetoclax, targeted TKIs).	Challenge: Reliance on older, often less effective chemo. Prohibitive cost of newer drugs. Erratic supply of even basic supportive care (blood products, specific antibiotics).
Infrastructure	Dedicated AL/Oncology Centers, Intensive Care Units, HCT (Transplant) capability.	Challenge: Few specialized centers. Lack of dedicated isolation rooms (Barrier Nursing). Insufficient specialized personnel (Hematologists, Pathologists).
Financing/Policy	Universal/Robust Health Insurance (NHIF in some LMICs exists, but coverage is partial).	Challenge: High Out-of-Pocket Expenditure (catastrophic financial burden). Diagnostics and monitoring often not covered.

KENYA'S PERSPECTIVE

- **Context:** Kenya is a lower-middle-income country with a significant patient burden. Actual burden not known due to no registry, diagnostics and high cost of treatment
- **The Triple Threat:**
  - **Diagnostic Delay:** Limited number of trained laboratory staff (clinical/hemo pathologists). Reliance on basic tests due to cost and limited availability of reagents/stains.
  - **Treatment Limitations:** Scarcity of targeted therapies and essential secondary anti-infectives. **No general availability of Radiotherapy or Stem Cell Transplant.**
  - **Financial Toxicity:** Even with the Social Hospital Insurance Fund (SHIF), coverage often *excludes* key diagnostics and long-term monitoring, leading to high abandonment rates and early death. *The policy is there, but the crucial fine print is missing.*
- **The Policy/Decision-Making Involvement Gap:** Clinicians and advocates have **limited direct input** into national drug procurement lists (essential medicines list) and the *scope* of insurance coverage, resulting in policies that don't match the clinical reality. Decisions are often top-down and budget-driven, not patient-outcome driven.

ACTIONABLE SOLUTIONS

- 1. **Strengthen Diagnostics** :
  - Establish Regional Molecular Diagnostic Hubs (centralized testing for multiple hospitals).
  - Public-Private Partnerships to subsidize the cost of essential leukemia diagnostics (e.g., flow cytometry, basic molecular markers). *If you can't see it, you can't treat it.*
- 2**. Optimize Treatment Protocols:**
  - Develop Context-Specific Guidelines (African-specific protocols) that optimize available affordable chemotherapy while integrating accessible targeted generics/biosimilars.
  - International Partnerships (Twinning Programs): Like the Moi Teaching and Referral Hospital (MTRH) program, for training and knowledge transfer, not just resource donation.
- 3**. Policy and Financing Advocacy:**
  - Advocate for SHA Reform: Policy must fully integrate *diagnostics, monitoring tests,* and full supportive care (barrier nursing, blood products) into the essential cancer benefit package.
  - Promote Affordability: Leverage mechanisms for generic/biosimilar procurement and encourage participation in global access programs.
  - Mandate Clinician Involvement: Create formal channels for hematologists and patient advocates to influence the Essential Medicines List and insurance benefit design. *The experts need a seat at the table, not just a suggestion box.*

CONCLUSION - A CALL TO ACTION

- Bridging the gap requires *policy innovation* as much as *medical innovation*. We need to move from **'care' as a privilege** to **'care' as a policy mandate**.
- **Need to have a forum to co-develop and share strategies.**
- **Final Thought:** Global health equity in acute leukemia care isn't just a moral imperative; it's an economic investment.

POWER IN PARTNERSHIPS

SETTING THE SCENE

- Median cost of new leukemia drugs: **>$150,000/year**
- Less than **5% of eligible cancer patients** enroll in clinical trials
- Leukemia has over **10 subtypes**—one-size-fits-all collaborations often fail to address this

DEBATE

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“Are current collaboration models truly benefiting patients and accelerating access in leukemia care?”

Collaborations with pharma *Do* Benefit Patients

Faster drug development: Pharma brings funding and resources that accelerate clinical trials and bring therapies to market faster.

Access to innovative treatments: Collaboration leads to compassionate use programs, early access schemes, and expanded access protocols for leukemia patients

Real-world evidence generation: Partnerships help collect post-marketing data, improving treatment protocols e.g. post-approval monitoring and registries helped refine patient selection criteria and manage side effects

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Collaborations with pharma *Don't* Truly Benefit All Patients

Profit-driven motives: Pharma may prioritize blockbuster drugs because they offer larger market potential over niche but needed leukemia subtypes e.g. T-cell ALL or BPDCN

Access inequality: After a leukemia drug is approved, companies may delay launch in certain countries due to pricing negotiations or lack of market agreement with local health systems

+ high cost of novel drugs (e.g. CAR-T) means not all patients benefit equally

Bias in research outcomes: Some pharma companies invest heavily in marketing drugs with limited clinical benefit, especially in cases where surrogate endpoints (e.g., response rate) are used instead of survival or quality-of-life measures. Patients may be placed on costly drugs that provide little real-world improvement, while better options may be underutilized.

Academic collaborations *Do* Benefit Patients

Cutting-edge research: Academia leads early-phase research and discoveries in biomarkers, resistance mechanisms, and novel therapies.

Multi-center clinical trials: Collaborative networks (e.g GIMEMA, EWALL, etc) allow for broad patient recruitment and faster results

Translational medicine: Close ties between bench science and clinical application mean patients benefit from faster innovation. Academic oversight ensures findings quickly inform clinical practice and future research

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Academic collaborations *Don't* Truly Benefit All Patients

Patient exclusion: Academic-led clinical trials often use strict inclusion/exclusion criteria, excluding elderly, frail, or comorbid patients — even though these groups make up a large portion of real-world leukemia cases e.g. AML trials excluding people over 75.

Higher burden on patients: Academic trial designs can be logistically burdensome — requiring frequent hospital visits, biopsies, or rigid timelines**.**

Academic silos: Institutions often compete for funding/data instead of sharing openly. Several large institutions (e.g., Memorial Sloan Kettering, Dana-Farber, MD Anderson) have developed their own proprietary leukemia mutation databases or platforms, but do not share full datasets due to intellectual property concerns or competitive advantage.

Collaboration with medical societies *Do* Benefit Patients

Rapid dissemination of guidelines: : New recommendations are shared widely through journals, online tools, and ASH's annual meeting.

Consensus building: Multidisciplinary panels ensure that care recommendations reflect best practices across settings - Panels include hematologists, oncologists, pathologists, pharmacists, and patient advocates

Educational initiatives: Guidelines are integrated into **continuing medical education (CME)** modules and webinars for practicing clinicians.

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Collaboration with medical societies *Don't* Truly Benefit All Patients

Lag in guideline updates: Guidelines may be slow to reflect emerging evidence e.g. a pivotal trial showing that a less toxic therapy is just as effective for older AML patients may be published, but official guidelines from ASH or NCCN remain unchanged for over a year.

One-size-fits-all approach: Guidelines may not account for patient diversity or global inequalities in access e.g. a guideline recommends molecular testing and targeted therapy for AML,but doesn't provide alternatives for regions where those tests or drugs are unavailable or unaffordable.

Influence of sponsors: Major societies like ASH or EHA depend on industry sponsorship for conferences, symposia, and publications, raising conflict-of-interest concerns e.g. a leukemia treatment symposium heavily sponsored by a drug company may disproportionately feature positive data about that company's drug, with less emphasis on side effects or alternatives

Collaborations with HCPs *Do* Benefit Patients

Improved patient education: Jointly developed resources help patients make informed decisions e.g. LLS work with physicians

Shared decision-making models: Patients are empowered to participate in treatment choices.

Clinical trial awareness: Patient organizations help match patients to trials they might not otherwise know about

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Collaborations with HCPs *Don't* Truly Benefit All Patients

Information overload or misalignment: Mixed messages can confuse rather than empower.

Tokenism: Patient voices may be included superficially rather than integrated meaningfully e.g. sometimes, HCPs focus on clinical metrics (e.g., remission rates), while patient groups emphasize quality-of-life outcomes — but these priorities aren't integrated in shared decision-making tools.

Limited outreach: Patient–HCP collaborations often focus on digitally connected, health-literate populations, missing marginalized groups e.g. a collaboration between a major leukemia organization and oncology societies launches an **online education portal** for clinical trials — but it is only in English, assumes internet access, and uses complex language

In summary

- Patients should be **at the center** of the debate
- Collaborations **must be inclusive, transparent, and patient-centered** to achieve real impact.
- There is a need for **global equity** in access—current models often favor high-income countries.
- Metrics for success should shift from publications/drug approvals to **real-world patient outcomes.**
- **Next-generation collaboration models** should truly translate to improved **access + outcomes**

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What would you change in the current collaboration landscape to make it truly patient-driven?

QUESTIONS TO ASK OURSELVES

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Acute Leukemia Advocates Network

- What is the biggest barrier to **effective collaboration** ?
- Are these collaborations prioritizing **speed** over **safety or affordability**?
- Do current models reflect the **needs of diverse patient populations**, especially in low-resource settings?
- How can we make sure that **patients are equal stakeholders** in all collaborative efforts?
- Are there **better models of collaboration** we should consider (e.g. public-private partnerships with strict access mandates)?

LESSONS FROM THE FIELD

ALAN
Acute Leukemia Advocates Network

Compassionate Use

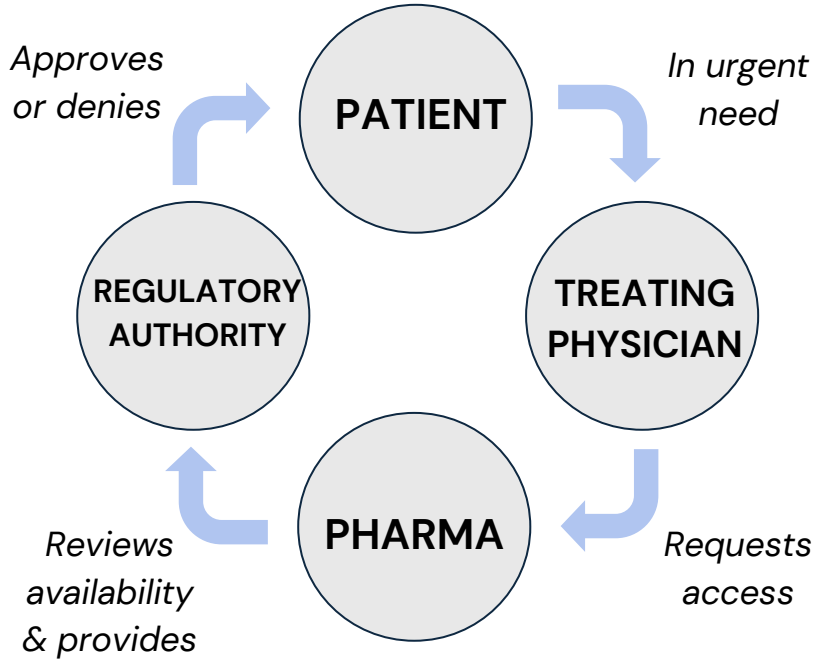
The Price of Life: Expanding Access to High-Cost Drugs in Kenya

Local Innovation to Bridge Gaps in New Zealand

What is Compassionate Use?

New treatments often exist, but are still in trials or pending approval.

Compassionate Use, also called Expanded Access = access to investigational drugs not yet approved or still in clinical trials.



A compassionate use journey

💪 Needed approval: ANSM, Gilead
💊 Drug concerned: Magrolimab

Understanding of the legal framework supported by patient organizations (ALAN, Anne Pierre, IconPLC) and ANSM experts

A compassionate use journey

💪 Needed approval: ANSM, Gilead
💊 Drug concerned: Magrolimab

Almost 2 months of process !!

Understanding of the legal framework supported by patient organizations (ALAN, Anne Pierre, IconPLC) and ANSM experts

Challenges: Food for thoughts

1

💭 Awareness & understanding

Many patients, carers & even physicians **don't know compassionate use exists**.

Namings & definitions can differ from one country to another.
EU Europe : Compassionate Use or Named-Patient Programs
US USA :Expanded Access
🌍 Others: Managed Access / Early Access Programs

2

🌎 Unequal access

Depends heavily on **country laws** (an american company does not perfectly understand French law for instance), on **hospital resources** and **physician awareness**

3

🗂️ Administrative complexity

Too **slow approval**

Unclear criteria to access compassionate use for physicians, carers and patients
Heavy process

4

🗣️ Patient & Physician voices

Limited integration in program design to raise awareness and co-creation.

Transparency on the data collected in the context of compassionate use.

The Price of Life: Expanding Access to High-Cost Drugs in Kenya

Balancing Innovation, Affordability, and Universal Health Coverage (UHC).

Elo Mapelu: Henzo Kenya and The Max Foundation

Kenya's Reality: High Cost, High Burden

- **Key Data Points:**
  - 80% of pharmaceutical spending is Out-of-Pocket (OOP). *(The biggest barrier to access is the patient's wallet.)*
  - 70% of medicines are imported, creating supply chain volatility and reliance on foreign pricing.
  - No Price Controls: Mark-ups are largely unregulated, pushing prices far above competitive procurement rates.
  - Highly fragmented supply chain, introducing multiple billing levels
- "UHC is impossible to achieve if the cost of treatment forces families into poverty."

Current Levers: Procurement & Local Production

- **Collective Buying (KEMSA)**
  - Mechanism: Kenya Medical Supplies Authority (KEMSA) uses bulk purchasing to secure lower prices for essential medicines.
  - Benefit: Provides subsidized/free medication in public facilities.
- **Local Manufacturing Goal**
  - Objective: Produce 50% of essential medicines locally by 2026.
  - Benefit: Reduces import costs, improves national supply security (A good defense against global crises).
- **Challenge:** Local market fragmentation and lack of a robust medicine pricing containment policy.

Global Negotiation: Leveraging Volume and Data

Strategy A: Pooled Procurement

- **Concept:** Countries group together (e.g., regional bodies) to maximize purchasing volume.
- **Wit:** *The pharmaceutical company now sees one giant buyer, not 10 small ones. They listen better.*

Strategy B: External Price Referencing

- **Concept:** Government sets a ceiling price based on what a drug costs in a basket of comparable countries.
- **Benefit:** Prevents the country from becoming a high-price outlier.

The East Africa Community Medicines Regulatory Harmonization Programme aims to facilitate access to safe, efficacious and quality essential medicines, through harmonization of regulatory requirements, guidelines, standards and tools for the EAC National Medicines Regulatory Authorities (NMRA).

EAC PARTNER STATES NMRAS

ABREMA

Pharmacy and Poisons Board

RWANDA FDA
Rwanda Food and Drugs Authority

DRUG AND FOOD CONTROL AUTHORITY
DFCA
SOUTH SUDAN

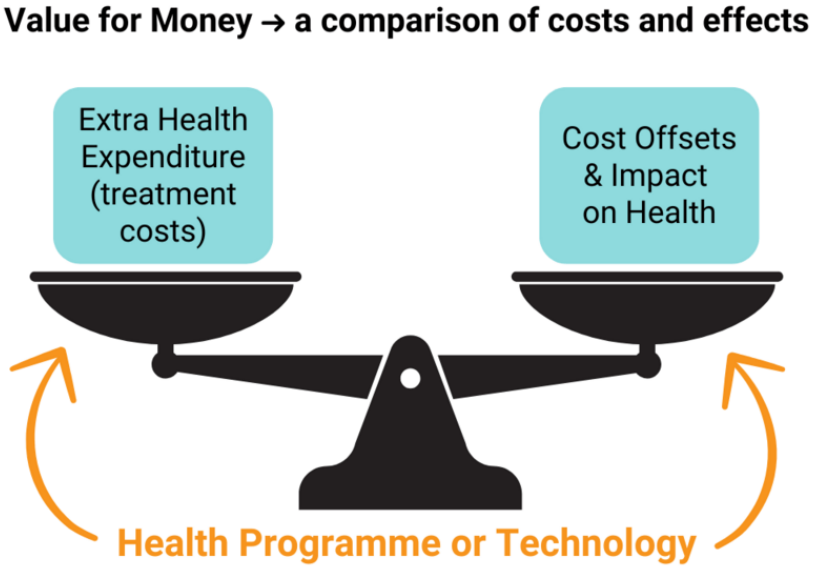
UGANDA
NATIONAL DRUG AUTHORITY
Safe Drugs Save Lives

TMDA
Tanzania Medicines & Medical Devices Authority

Zanzibar Food & Drug Agency
ZFDA

Paying for Value, Not Just Price Tag

- **Mechanism 1: Health Technology Assessment (HTA)**
  - **Function:** Systematically evaluates the **clinical benefit and cost-effectiveness** of a drug.
  - **Outcome:** Used to decide if a high-cost drug is worth covering on the national essential medicines list.
- **Mechanism 2: Managed Entry Agreements (MEAs)**
  - **Concept:** Confidential, flexible agreements where the manufacturer gives rebates or discounts tied to volume or patient outcome.
  - **Example:** A **Risk-Sharing Agreement** where the government only pays in full if the patient responds well.



Intellectual Property:

- **Tool A: Promoting Generics/Biosimilars**
  - **Action:** Rapid regulatory approval for generic versions upon patent expiration to create immediate price competition.
- **Tool B: Compulsory Licensing**
  - **Action:** Legal provision that allows a country to authorize a local entity to produce a patented drug **without the patent holder's permission** (usually for public health crises, like pandemics or severe disease burdens).
  - **Impact:** A credible threat that forces the manufacturer to the negotiating table with a much better offer.

Summary:

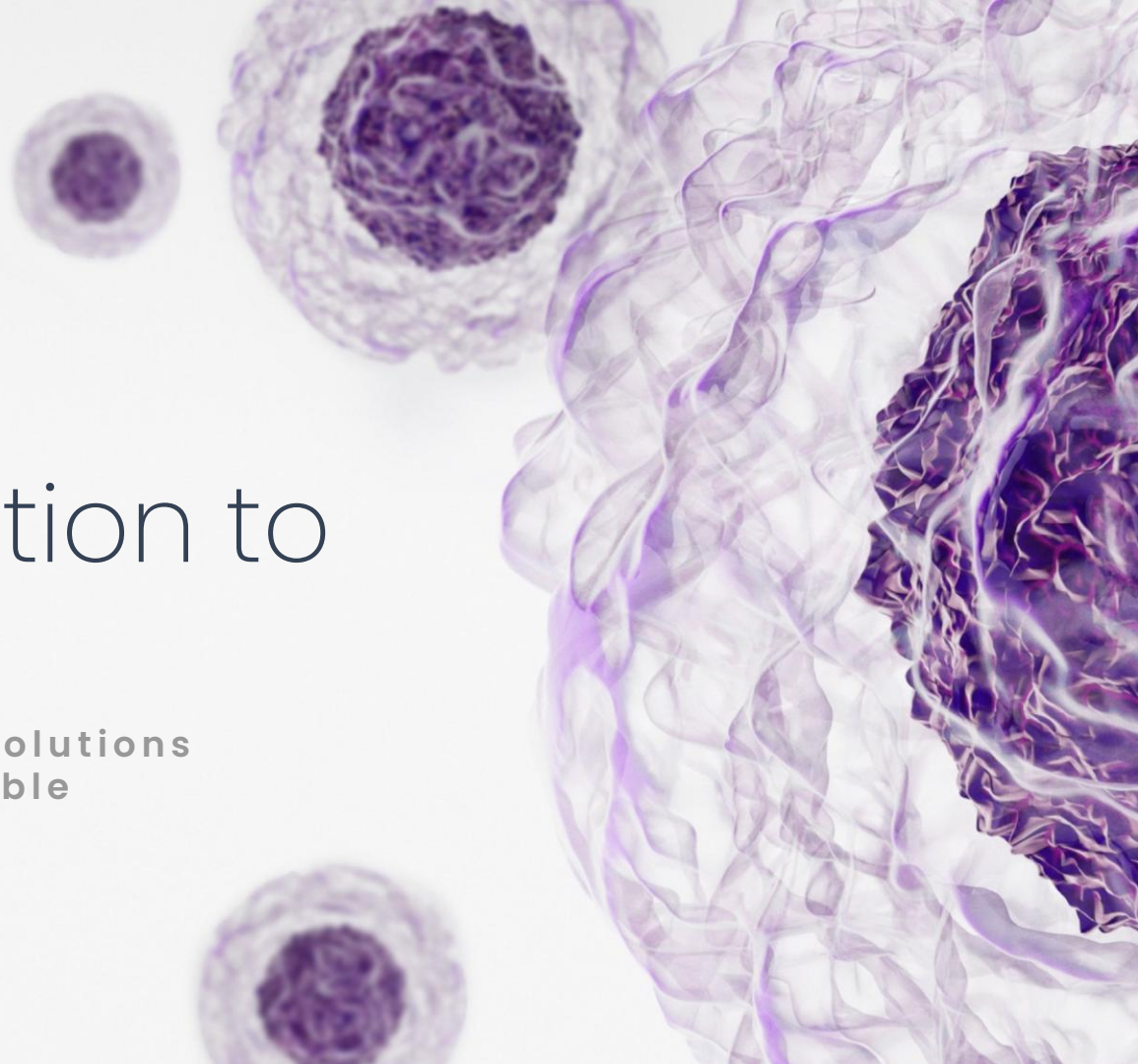
- **Key Takeaways (The 3 Cs):**
  - **Contain:** Implement domestic policies to regulate and contain supply chain mark-ups (solving the Kenyan *last-mile* problem).
  - **Concentrate:** Use centralized/pooled procurement to concentrate buying power (KEMSA and regional collaboration).
  - **Challenge:** Leverage HTA and Intellectual Properties flexibilities to challenge unfair pricing and only pay for proven value.

BioOra

CELL THERAPY FOR EVERY PATIENT IN NEED

Local Innovation to Bridge Gaps

CAR T-cell manufacturing solutions for faster and more affordable access



BioOra Company Overview

NEW ZEALAND BASED

New Zealand-based biotech company, headquartered in Wellington, 35 staff, CAR-T manufactured in-house in NZ

JOINT VENTURE

Between Malaghan Institute of Medical Research (MIMR) and Bridgewest Ventures

NEXT-GENERATION CAR-T

Developing a portfolio of therapies with safety profile allowing for outpatient administration

AUTOMATED MANUFACTURING

Validated manufacturing platform, enabling cost-effective commercial scale production

Manufacturing Innovation

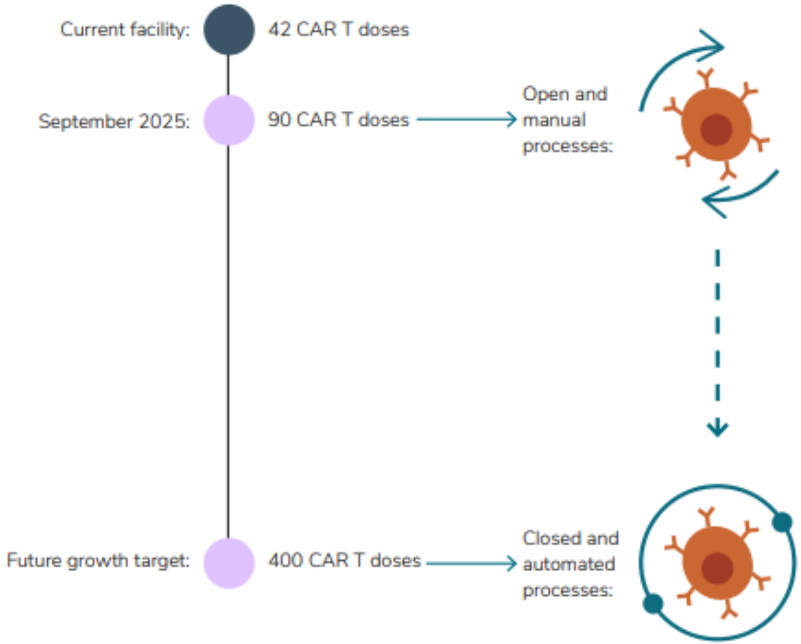
Automated Production

- Technology: Lonza Cocoon® Platform (closed-system)
- Timeline: 11-day production process
- Vein-to-vein: Median 49 days (range 34-189)

Quality & Capacity

- Medsafe manufacturing audit completed January 2025
- Currently supporting ENABLE-2 across 3 sites
- Commercial facility planned in Christchurch (mid-2026)
- Significantly reduced costs vs manual manufacturing

Capacity to Scale



BioOra

CELL THERAPY FOR EVERY PATIENT IN NEED

WTZL-002: Third Generation

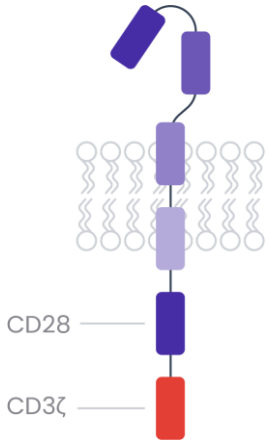
CD28 + TLR2 + CD3ζ costimulatory domains

Interposition of TLR2 domain between CD28 and CD3ζ

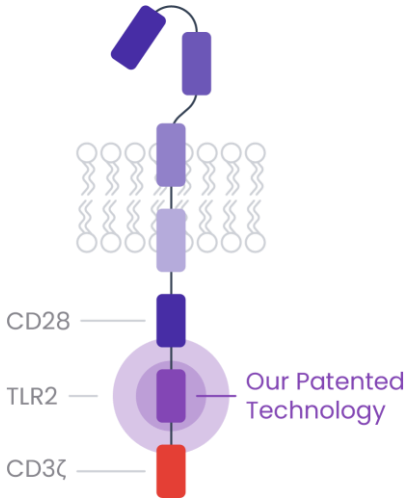
Scientific Rationale for TLR2 Domain

- GM-CSF and IFN-γ are associated with CRS and ICANS
- TLR2 domain reduces production of toxicity-associated cytokines
- Maintains CAR T-cell cytotoxicity while lowering adverse events
- Enhanced T-cell expansion, modulated cytokines, promotes long-lived memory

2nd GEN
FDA Approved Therapy



3rd GEN
Atla-cel



Novel Third-Gen CD19-Directed CAR-T

Novel CD19-CAR-T construct with low rates of CRS/ICANS, enabling outpatient delivery

50 patients total dosed to date

53% COMPLETE RESPONSE

Comparable to commercial CART products

0% SEVERE ICANS

3% Grade 1 / 2

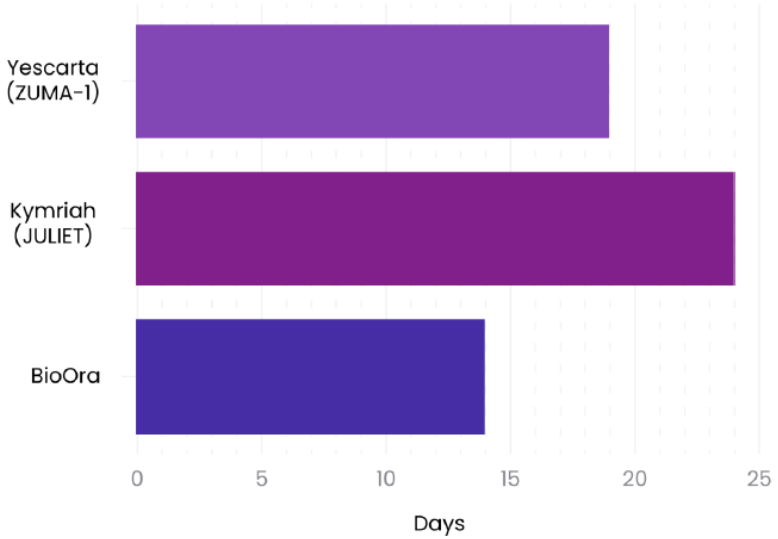
11-DAY TURNAROUND

Fast automated manufacturing

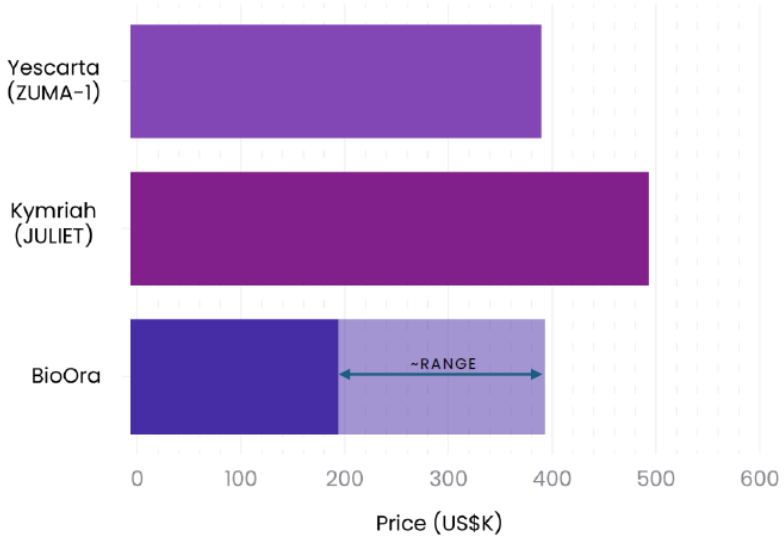
Outperforming on Time and Cost

BIOORA OFFERS A FASTER, MORE COST-EFFECTIVE PATH TO TREATMENT

Time to Manufacture



Cost to Manufacture



ENABLE-2 Trial Progress

- Study start-up Jul 2024 (Wellington)
- First patient enrolled Aug 2024
- Auckland and Christchurch sites activated Feb 2025
- Manufacturing scale-up Jul 2025
- Toxicity and efficacy profile consistent with ENABLE-1 data
- Anticipated end of enrolment Aug 2026

What Next?

- Submissions to Medsafe/PHARMAC due 2026, potential approvals 2027
- Plans for additional clinical trials in other indications
- Plans for other regions in APAC


Bioora
www.bioora.com

BREAK – BE BACK AT 11:15 AM !

Buddy Check

- Reconnect with your buddy
- Reflections
  https://www.menti.com/alabhtx67bbm
- Maybe a last picture ? **#ALANsummit**

WORKSHOP:
WHAT TO ADVOCATE FOR LOCALLY?

#ALANsummit

- **45 min**
- **Individual reflections**
- **When completed, take a picture or scan it and send via email to Samantha**

STAYING ENGAGED & EXPANDING IMPACT

Open mic

From all the nuggets we brought you, what treasure are you bringing back home ? and why ?

Thank You to Our Sponsors

ALAN
Acute Leukemia Advocates Network

We would like to extend our sincerest thanks to the following sponsors for their invaluable support and partnership:

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Otsuka Pharmaceutical, S.A.

SENTI BIO SERVIER Syndax Takeda ThermoFisher SCIENTIFIC

THANK YOU TO ALL OF YOU !

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Ce n’est qu’un au revoir !