

# Boosting the Impact of Patient Involvement in Drug Discovery

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# Why should we involve patients in drug discovery & development?

The right stakeholder



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Making choices



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Making choices

Patient Engagement is booming internationally

Changing healthcare landscape



**NICE** National Institute for Health and Care Excellence



More connected & demanding patients



Involving patients: reduces costs

## Return on Investment

Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project

[Sennell Leites, MD, PhD, Kenneth Cox, MBA, Eric L. Eisenstein, DBA](#)

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For a pre-phase 2 project, the cumulative impact of a patient engagement activity that avoids one protocol amendment and improves enrollment, adherence, and retention is an increase in net present value (NPV) of \$62MM (\$65MM for pre-phase 3) and an increase in ENPV of \$35MM (\$75MM for pre-phase 3). Compared with an investment of \$100,000 in patient engagement, the NPV and ENPV increases can exceed 500-fold the investment. This ENPV increase is the equivalent of accelerating a pre-phase 2 product launch by 2½ years (1½ years for pre-phase 3).



**So,  
what are we  
waiting for?**



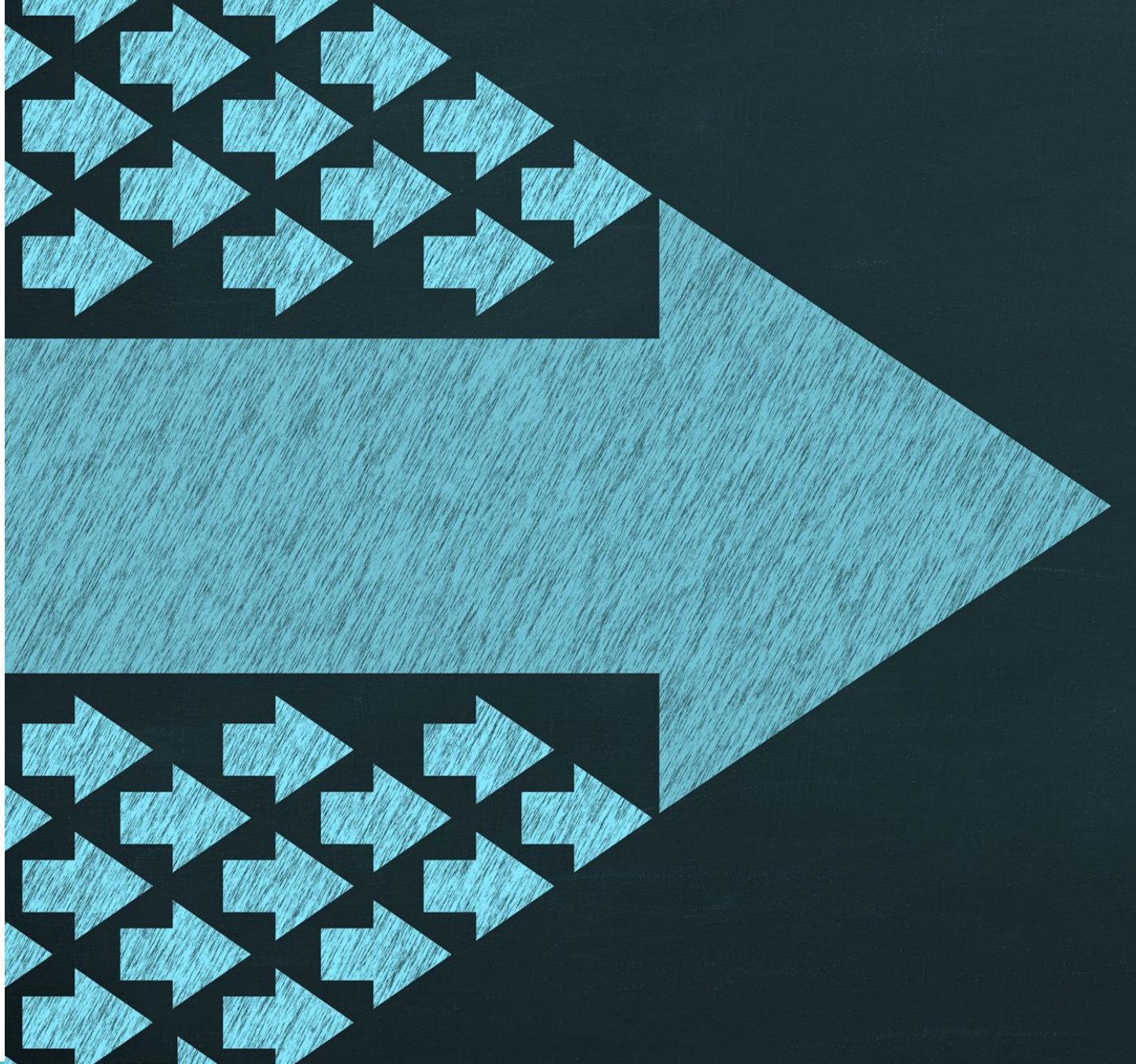
# Barriers, Limitations & Cold feet

| Category                                                                                 | Challenges                                                                                                                                     | Key concerns                                                                                                                                                                        |
|------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>Lack of Mutual Trust &amp; Understanding</b>                                          | Differences in expectations between POs and pharmaceutical companies.                                                                          | Concerns about transparency and balanced representation.                                                                                                                            |
| Doubts About Effectiveness & Distrust in Previous Initiatives                            | Past collaborations have not always been successful.                                                                                           | Concern that this initiative may not add real value or result in tangible outcomes.                                                                                                 |
| <b>Funding Challenges &amp; Limited Capacity</b> to Engage                               | Many POs have limited financial resources, and most operate with small teams, often made up of individuals personally affected by the illness. | Unclear how long-term funding will be secured (cash or in-kind).<br>Risk of overburdening staff and volunteers, who may already have limited time due to the nature of the illness. |
| <b>Internal Resistance</b> Within POs and Pharma & <b>Commercial Interests</b> of Pharma | Not all POs are open to working with the industry.                                                                                             | Fear that collaboration may serve corporate PR rather than patient interests.                                                                                                       |
| Long-Term Commitment                                                                     | Uncertainty about sustained engagement from all stakeholders.                                                                                  | Risk of the initiative losing momentum over time.                                                                                                                                   |



# From a huge challenge to a solid collaboration

Who takes the lead  
when you co-create?



# Who takes the lead when you co-create?

Independent  
facilitator

lygature pioneering medicine.  
together.

Exploration Phase

- Interviews
- Desk research
- International initiatives



PATIENT FOCUSED  
MEDICINES DEVELOPMENT

Defining the scope

- Netherlands
- Clear guidelines
- Topics like EU HTA, sustainability, shortages, low literacy,...



# Exploration... → What's next?

## Goal: Build trust and commitment

- Discuss and confirm **urgency and momentum**.
- Exchange knowledge and experiences, including both good and bad practices.
- Have an open conversation about **mutual interests and expectations**.
- Define the scope and decide which topics will take priority.
- Establish a collaboration framework (governance, funding, involved parties).

## How: Co-Creation of the Collaboration Initiative

- **Equal involvement** of POs, pharmaceutical companies, and other stakeholders.
- A neutral facilitator leading discussions and promoting trust.
- A **clear governance** structure in which POs have at least 50% of the influence.
- **Transparency and feedback** as fundamental principles.
- Achieve small, concrete successes to strengthen trust.

**Proposed Foundation Phase (12 months)**

# Next year: Same time, Same place, with Success?!

- Mutual trust
- Neutral structure
- Equal partners
- Co-creation
- Active participation
- Concrete joint project: euHTA regulation

→ **Successful sustainable collaboration**



# Organizations involved in exploration phase

- ReumaZorg Nederland (Arthritis Care Netherlands)
- Stichting Pulmonale Hypertensie (Foundation Pulmonary Hypertension)
- Diabetesvereniging Nederland (Dutch Diabetes Association)
- Nierpatiënten Vereniging Nederland (Kidney Patient Association)
- Stichting 1op5 (Foundation for rare cancers)
- Stichting Melanoom (Melanoma Foundation)
- VIG (Vereniging Innovatieve Geneesmiddelen)
- AstraZeneca
- Johnson & Johnson
- Pfizer
- Boeringer-Ingelheim
- Lygature
- PFMD



# Thank you

Questions?