
# Aligning Cardiac Safety Studies With Emerging Trends in Product Development

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# Increased Focus on Rare-Disease Drug Development

- Simpler and swifter product development and commercialization.
- Longer periods of market exclusivity.
- Ability to command premium pricing.

| Strategy[1] | Example |
|---|---|
| **Partnership formation** | • GSK's partnership with Amicus (Amigal for Fabry's Disease). |
| **Creation of new business units** | • Pfizer and GSK's dedicated rare diseases units. |
| **Restructuring of R&D** | • Novartis's NIBR: "Pathway-driven" approach to R&D.<br>• **Proof-of-concept → Indication expansion.** |

- **How can this emerging product development strategy create new opportunities for cardiac safety testing outside of traditional clinical trials?**

[1]Evolving Strategies for Rare-Disease Drug Development, Decision Resources LLC. *Spectrum*.


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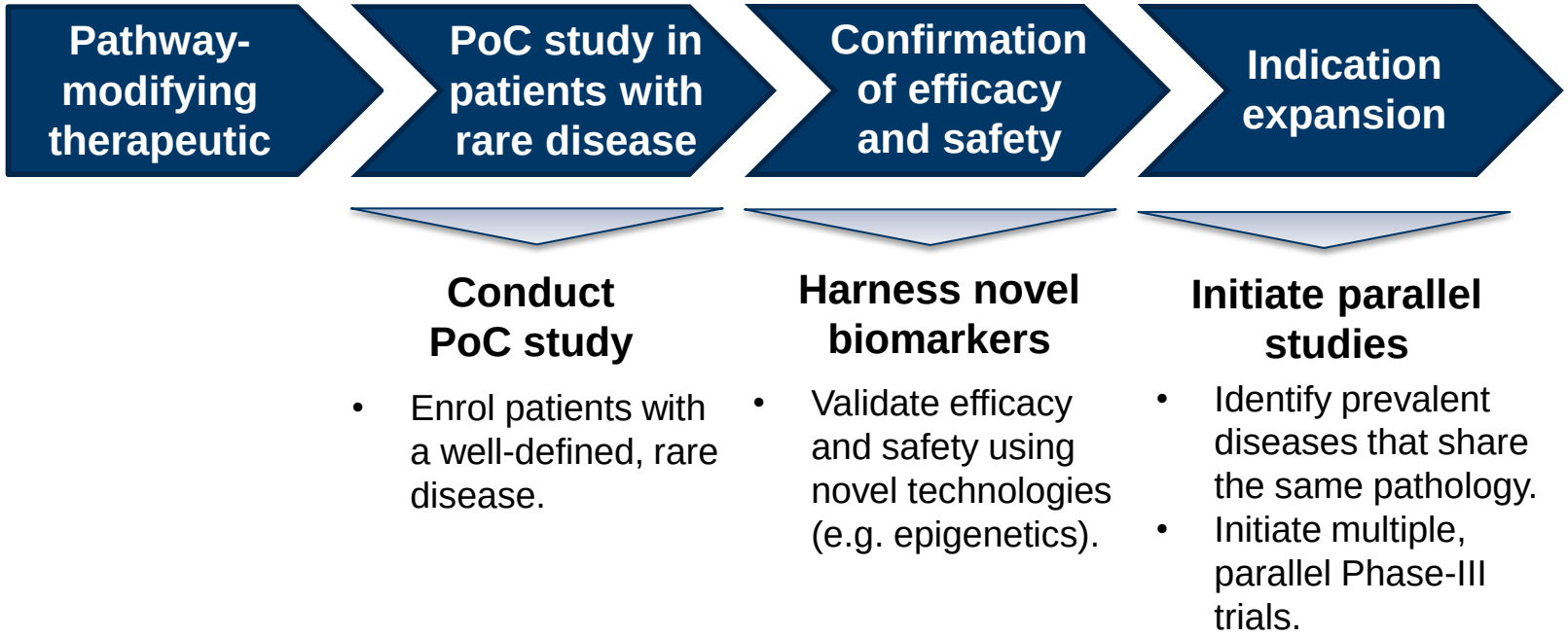
# Proof-of-Concept Studies and Indication Expansion: An Opportunity for Cardiac Safety?

- Approach pioneered by the NIBR.
- Repurposing of rare disease therapies for more prevalent conditions that share a common underlying pathology.
- Indication expansion maximizes the ROI.




- **Can we optimize cardiac safety assessments in rare disease PoC trials prior to indication expansion?**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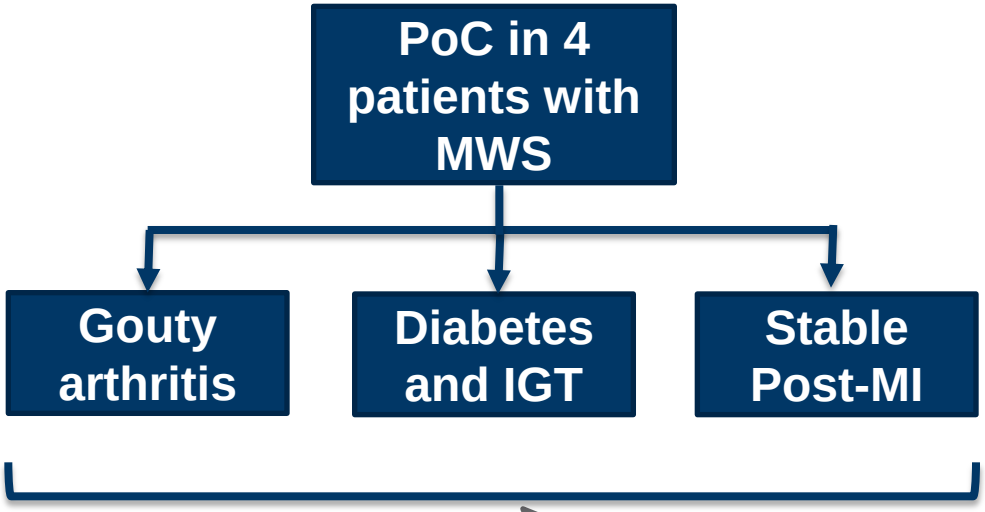
# Case Study: Novartis's Ilaris (Canakinumab)

- Monoclonal antibody targeting IL-1β.
- Approved in 2009 for CAPS; 2010 sales of $29 million.
- Successful transition from rare disease indication to more prevalent conditions.




Forecasted peak year sales (all indications) of $500 million - $1 billion.

- How can we further confirm the cardiac safety of early stage therapies prior to expansion into prevalent indications?
- What biomarkers of CV toxicity can we use?
- How will patient attributes shape cardiac safety data?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# Innovating the Proof-of-Concept Study for Cardiac Safety

- How can cardiac safety testing be optimized for "truth-seeking" vs. "success-seeking" new product development strategies?[2]

**Expedited PoCs**

- Eli Lilly's Chorus: Dedicated, "truth-seeking" PoC unit.
- Work limited to core clinical experiments.
- Mean time to PoC is 29 months.
- Mean cost per molecule is $6 million vs. $15-32 million (industry benchmark data).

- **What opportunities exist for incorporating cutting-edge cardiac safety analyses into expedited PoC studies?**
- **Can the increased use of novel cardiac safety biomarkers in expedited PoC studies further de-risk the late-stage pipeline?**

[2]A More Rational Approach to New-Product Development. *Harvard Business Review*, 2008.


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# Where Do We Go From Here?

- Emerging product development trends create new avenues for cardiac safety testing.

- Lean PoC studies can provide a wealth of critical data prior to indication expansion in parallel trials (e.g. NIBR's approach) and full-scale clinical development (e.g. Eli Lilly/Chorus's approach).

- Can "expedited" PoC studies be optimized, by the integration of novel cardiac safety biomarkers, to enhance decision making and limit late-stage failures?

- What knowledge and methodologies from large-scale trials can we transfer to "expedited" PoC studies?


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