Summary Report: Pre-Clinical Therapy Development in Canada 2025 Workshop

Summary

On April 10, 2025, RareKids-CAN and the Canadian Rare Disease Network (CRDN) co-hosted a mini-workshop alongside the RareKids-CAN Conference in Ottawa. The session, titled *"Pre-clinical Therapy Development in Canada: Who, What, How, and Where Do We Go From Here?"*, was seen as especially timely given the shifting global geopolitical landscape, which is underscoring the need for stronger domestic research capacity, supply chain resilience, and national leadership in rare disease (RD) innovation. Attendees at the event included a subset of individuals invited to the RareKids-CAN annual conference—primarily researchers conducting pediatric advanced therapeutics clinical trials, members of the RKC Executive Committee, and select representatives from Canadian pre-clinical manufacturing facilities (see below).

The workshop featured a keynote by Dr. Eric Hoffman and initial discussions facilitated by Dr. Leanne Ward to explore current barriers, emerging opportunities, and early priorities for building a coordinated national approach. While the dialogue was productive, organizers acknowledged that due to funding limitations and the political restrictions with the election, some essential key individuals—such as regulators, manufacturers, funders, and early-stage therapy developers—were not in attendance to participate in the conversation. Thus, this report captures preliminary insights rather than comprehensive conclusions.

Key Themes and Takeaways

Regulatory Strategy and Approval Pathways

- **Health Canada challenges**: Current Health Canada processes lack clarity and are not sufficiently tailored to support academic or non-commercial developers. There is a disconnect between regulatory pathways designed for industry and those for academic- and early-stage innovators.
- **Early regulatory integration is key:** Involvement of regulatory bodies (e.g., Health Canada, FDA) early in the development process - especially on Chemistry, Manufacturing, and Controls (CMC), Quality assurance/ quality control (QA/QC), and pre-IND meetings (such as with the FDA's Emerging Technology Program or ETF) - was noted as critical for reducing downstream issues and streamlining approvals. Validated bioassays and high-quality pre-clinical data were noted as key enablers.
- **Leveraging US approval and export-oriented planning**: The U.S.'s FDA accelerated pathways were mentioned as benchmarks, with participants suggesting that Canadian developers could benefit from aligning with these mechanisms. In some cases, pursuing FDA approval may offer faster pathways to market compared to domestic routes.

Hence, regulatory and manufacturing planning should start with end markets in mind (e.g., FDA standards if exporting to US). Exporting therapies to the U.S. could be a strategic route for Canadian-developed therapies.

- **Global alignment and harmonization**: Canada could learn from other countries like China, which is building infrastructure to become a drug exporter (not there yet). Canada could identify its unique strengths to do the same. Regulatory standards are largely aligned between Canada, Japan, and Europe, suggesting the potential to develop products that meet multiple jurisdictional requirements. It was noted that this also represents an opportunity to develop internationally harmonized SOPs.
- **Innovative trial designs**: N-of-1 and off-label use protocols must be supported by structured ethical, consent, data-sharing, and adverse event frameworks. A national committee (e.g., for innovative therapies) was suggested to support innovative trial pathways.
- **Incentives & de-risking**: Reducing safety risks, proving feasibility, and early regulatory planning were noted to be attractive to funders. Opportunities such as orphan vouchers were raised as useful tools in de-risking and accelerating development. Long-term funding strategies are essential to bridge the high costs of regulatory and post-market phases.

Infrastructure, Capacity, and Translation Gaps

- **Academic strengths**: Canada's academic infrastructure is strong, particularly due to its disease-specific expertise that must be preserved or partnered with, especially as CROs and commercial partners are lacking this expertise/knowledge. However, coordination across pre-clinical, clinical trial networks, and manufacturing capabilities is lacking, which can lead to duplication and inefficiency.
- **Natural history data is foundational**: Natural history studies are essential for clinical trial design, regulatory engagement, and endpoint development. These must be embedded early in therapy development pipelines.
- **Pre-clinical to clinical gap**: Often a “valley of death” where science exists, but transition is stalled due to lack of champions, funding, or operational readiness. High failure rates in Phase I are more often linked to systematic and logistical barriers (e.g., patient recruitment/retention, data readiness, and regulatory complexity) than scientific shortcomings—a critical insight for system design.
- **GMP and manufacturing challenges**: Manufacturing planning must start early—many teams struggle to find GMP facilities and manufacturing expertise (especially for biologics and gene/cell therapies) or understand costing implications which hinders progress. A national directory of facilities and costing tools could support better planning and more efficient partnerships.
- **Scalable platform technologies:** Development of scalable and adaptable platforms (e.g., ASOs, CAR-T) that can address multiple conditions was encouraged. This approach can reduce regulatory and development burdens. Rather than committing to single-disease pipelines, flexible disease-specific advisory committees should guide development across conditions.

- **Coding and identification issues:** RD patients are often invisible in health systems due to inconsistent coding and data fragmentation. Improved coding is needed to understand healthcare utilization and readiness.
- **IP & Commercialization:** Participants emphasized keeping IP (often owned by university or investigator) and potential revenues within the public or academic system where possible and for as long as possible (as opposed to commercial licensing too early). Institutional innovation offices and support for academic spin-offs (e.g., seed funding, legal support, IP) are critical for moving discoveries into development pipelines.

Patient-Centric Development

- **Patient & family involvement:** Strong emphasis was placed on patient involvement across all stages of therapy development, from defining relevant clinical outcomes to supporting recruitment. Ethical considerations remain paramount. While EMR systems are poor at identifying rare disease patients, patients can be reached via trusted organizations and social media with appropriate consent (good example is DMD where well-organized patient organization and desperate situation enabled success).
- **Decentralized trials:** Therapies and trials should minimize patient burden—ideally allowing for home-based or local treatment options and avoiding travel for those living far from urban centres. Strong connections with patient organizations improve recruitment, retention, and outcomes.
- **Registries and digital tools**: National contact and re-contact registries (e.g., AllforOne-Connect) are essential tools. A national, digital re-contact registry—integrated with EMRs and allowing app-based e-consent—could improve patient recruitment and engagement, increasing efficiency, standardization, and patient engagement.

Coordination, Knowledge Sharing, & Next Steps

- **Toward a national "playbook" or collaborative hub**: Participants proposed creating a national hub or national playbook for RD therapy development (similar to BioCanRx) – a central resource compiling regulatory pathways, manufacturing resources, funding resources, and best practices. As part of this or as an initial step, a centralized advisory team or network could be established to offer guidance to academic or early-stage developers across key areas (CMC, regulatory strategy, clinical, IP, manufacturing, and more).
- **Institutional memory & national mapping**: It was noted that a lack of institutional memory, reusable templates, SOPs, and process documentation (e.g., lessons learned) across projects results in duplication and inefficiencies. It's important to capture knowledge across projects and partners (e.g., through tools like Project Zero or perhaps RareKids-CAN). In addition, a centralized national map of expertise, GMP facilities, patient registries, expertise and capabilities is needed to help coordinate efforts.
- **Build momentum with ready projects**: Focus on projects poised to move forward and capable of demonstrating impact quickly to build credibility and attract investment. Projects like Popcorn or Bernard's program were mentioned as candidates. Focusing on

specific modalities (e.g., ASOs, CAR-T) where Canada has existing infrastructure or leadership may be strategic to get early wins and attract investment.
- **Strategic public investment is needed**: Long-term funding strategies are needed - suggestions included ISED-like applications, the Strategic Science Fund, or leveraging SOIP or D2R mechanisms for RD-focused product development.

Next Steps

As a starting point, it was agreed that conducting an environmental scan of existing GMP facilities across Canada would be valuable. This scan should focus on identifying the services currently offered by these facilities to better understand the national pre-clinical infrastructure and capabilities.

In addition, there was consensus on the need to convene a more comprehensive workshop in the future. This session would bring together stakeholders involved in the full pre-clinical development continuum—from identifying unmet needs and conducting animal studies to assembling regulatory submission packages for Health Canada in preparation for human clinical trials.