N=1 COLLABORATIVE

# Strategic Plan: 2026-2029

Advances in genetic medicine have created the possibility of developing individualized therapies tailored to a patient's unique genetic variant. This emerging practice, known as Interventional Genetics, represents a new model for treating genetic disease.

Yet turning this promise into scalable practice remains a challenge. Efforts across the ecosystem are fragmented, limiting the shared knowledge, data, collaboration, and infrastructure needed to move from individual successes to practice that can reach more patients.

Founded in 2021 as a 501(c)(3) nonprofit organization, the N=1 Collaborative (N1C) was created to address this challenge globally. As the professional home for Interventional Genetics, we convene and connect physicians, scientists, drug developers, parents, and others to exchange knowledge, collaborate, advance shared standards, and build community-driven solutions that no single organization can develop alone.

This Strategic Plan establishes N1C's priorities for the next three years, translating our mission into focused objectives and initiatives that guide decision-making, resource allocation, and program execution.

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# Mission

**N1C is the professional home for interventional genetics. We enable the ecosystem by convening and connecting practitioners and developers to exchange knowledge, collaborate, set the tone for responsible innovation, and guide the standards that advance the field.**

To underscore our mission, we specify:

**Practitioners** include those involved in the assessment, development, administration, and delivery of individualized genetic interventions, including clinicians, scientists, and patient organizations.

**Developers** include those responsible for creating individualized therapies, including academic institutions, nonprofit and advocacy organizations, biotechnology companies, and pharmaceutical companies operating within the pre-competitive ecosystem.

**Standards** include best practices, frameworks, and guidance that support the responsible development and delivery of individualized medicines. N1C helps advance these standards through community convening, expert consensus, publications, and knowledge sharing.

# Vision

**N1C envisions a world where individualized treatments for patients with genetic disease are globally accessible at scale; they are developed responsibly, with urgency, and enabled by shared learnings.**

This vision reflects our shared understanding that:

- **Patients are at the center of our work.** The field is moving beyond traditional distinctions based on disease prevalence. N1C's vision reflects an individualized approach that begins with the patient.
- **Together, we advance global accessibility at scale.** Interventional genetics has demonstrated what is possible for individual patients. Our definition of success is a future in which these approaches become equitable and accessible to patients worldwide.
- **Rigor & Urgency are a dual imperative.** The field must advance efficiently to meet the needs of patients facing significant medical needs today. At the same time, progress must be grounded in scientific rigor, patient safety, and a shared architecture of trust.

# Values

**N1C recognizes a responsibility not only to accelerate progress, but to shape how progress occurs.** These values guide our decisions, define our culture, and establish the principles we believe are essential to building a responsible and sustainable future for interventional genetics.

- **Open Collaboration**: Knowledge and data shared within the N1C ecosystem should benefit the entire community. We foster open exchange, reduce silos, and promote the dissemination of insights that can help practitioners develop and deliver treatments more effectively.
- **Ethical Integrity**: We are committed to advancing the field in a manner that prioritizes patients, scientific rigor, and public trust.
- **Neutrality**: N1C serves as an independent convening ground where diverse stakeholders can collaborate. We maintain our neutrality by ensuring that no single institution or individual determines the direction of the community or its work.
- **Collective Progress**: We believe the greatest advances occur when success is shared across the ecosystem. N1C measures impact by the growth, capability, and outcomes enabled through collaboration and shared infrastructure.
- **Shared, Iterative Learning**: Interventional genetics is an emerging discipline, and continual learning is essential to progress. We embrace new evidence, share lessons learned, and refine our approaches as knowledge evolves.

# How We Advance Our Mission

N1C advances its mission through three interconnected pillars that strengthen coordination across the interventional genetics ecosystem.

**Sharing Knowledge**

Eight N1C Workgroups
Seminar & Podcast Series
Annual Meeting

**Sharing Resources**

Donated Resource Center
Connections platform

**Sharing Data**

Gene Registry
Preclinical Database
Clinical Database

**Knowledge & Tools:** Eight cross-sector workgroups develop practical tools, consensus guidance, and shared resources that advance best practices across the field.

**Shared Platforms:** N1C's platforms connect stakeholders, increase program visibility, and expand access to critical resources. Together, the Connections platform, Gene Registry, and Donated Resource Center strengthen coordination across the ecosystem.

**Education & Convening:** Seminars, podcasts, and workshops provide open-access education through technical deep dives, case studies, and cross-sector perspectives from across the field.

**The Annual Meeting** brings together scientists, clinicians, families, industry, and regulators to share knowledge, align on emerging priorities, and accelerate progress toward scalable individualized medicines.

# Theory of Change

If N1C transforms a fragmented ecosystem into a connected learning system —where practitioners are convened, supported with shared infrastructure and knowledge, and guided by common standards of evidence and ethics…

Then the field shifts from isolated, one-off efforts to a compounding model of development, where insights, data, and approaches are continuously reused and improved—unlocking safer, faster, and more scalable delivery of individualized therapies.

# Strategic Arenas

Over the next three years, N1C will focus its efforts across four strategic arenas designed to strengthen the interventional genetics ecosystem and accelerate access to individualized treatments globally.

| Strategic Arena | Objectives | Impact |
|---|---|---|
| **Knowledge Leadership** | Deliver trusted, timely knowledge exchange through the Annual Meeting, Seminar Series, and Connections. | The field moves faster because knowledge flows freely across institutions, disciplines, and geographies. |
| **Professional Practice** | Develop the programs, infrastructure, and standards that advance the practice of Interventional Genetics. | N1C transforms distributed expertise into shared resources. |
| **Strategic Alliances** | Align partners across academia, industry, funders, and government. | The field operates in concert rather than in parallel. |
| **Institutional Foundation** | Strengthen leadership, governance, and financial systems to support long-term sustainability. | The field is supported by an institution with the people, capacity, and resources to serve it — now and as it grows. |
| **Outside our scope** | N1C does not develop, fund, or administer therapeutic programs. | |

# By 2029

The following outlines what success looks like for each arena at the end of this plan's three-year horizon

**Knowledge Leadership**

- N1C's Annual Meeting, Seminar Series, and Connections platform serve among the field's primary venues for timely knowledge exchange, with sustained growth in participation
- N1C's publications, programming, and expert perspectives help shape scientific, clinical, funding, and policy discussions in interventional genetics
- N1C contributes programming at external scientific society meetings, extending field knowledge beyond its immediate network

**Professional Practice**

- N1C Workgroups continue to convene practitioners to develop standards and tools that evolve alongside the field
- N1C resources including consensus guidelines, coordination frameworks, and the Roadmap to Treatment, are shaped with and adopted by institutions across the field
- N1C's shared infrastructure including Connections, the Donated Resource Center, databases, and the Actionability Coordination Center are actively used by practitioners and developers internationally
- N1C recognizes and develops the field's practitioners through training, mentorship, and annual awards

**Strategic Alliances**

- N1C establishes durable, active collaborations across academia, industry, government, and international partners
- Industry partners collaborate through N1C on shared, pre-competitive priorities

**Institutional Foundation**

- A diversified, unrestricted revenue base with multiple funding streams contributing meaningfully
- A board with the expertise and structure to guide N1C through its next stage of growth
- An operating model that enables consistent, reliable execution as N1C grows