

IRDiRC Goals, by 2027

- ▶ All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently undiagnosable individuals will enter a globally coordinated diagnostic and research pipeline
- ▶ 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options
- ▶ Methodologies will be developed to assess the impact of diagnoses and therapies on rare diseases patients