

## Operating Committee

### IRDiRC Goals 2017-2027

1 March 2017

#### IRDiRC Goals – For Vote by the CA

*N.B., in the survey, each Vision/Goal will have a click box: (1) Approve, as is; (2) Approve with Option. Each will also include a comment box.*

- ▶ **Vision:** Enable all people living with a rare disease to receive diagnosis, care, and available therapy within one year of coming to subspecialty medical attention
  - **OPTION:** add “in an environment that incorporates care and research”
  
- ▶ **Goal 1:** Diagnosis of all patients having a previously identified RD will occur within 1 year of coming to subspecialty medical attention, and all currently undiagnosable cases will enter a globally coordinated diagnostic pipeline
  
- ▶ **Goal 2:** 1000 new therapies for rare diseases will have been approved
  - **OPTION:** change to “therapies (drugs and devices)”
  - With a majority being first treatment for a disease
  - And standards for IRDiRC recognized non-regulated interventions will be defined
  
- ▶ **Goal 3:** All rare disease patients will receive available treatment within 1 year of diagnosis
  - **OPTION 1:** omit Goal 3 entirely since already included in the vision
  - **OPTION 2:** change to “Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients”