IRDiRCs Current State of Progress-Summary
Number of New Orphan Drugs

- Monthly update of number of new indications for orphan diseases
- Source: EMA and FDA

Updated on www.irdirc.org
Number of Identified Genes Causing Rare Diseases

Cumulative number of genes linked to rare diseases by year since 2010

Source: Orphanet Data
Actionables to ensure IRDiRC meets its objectives for the rare diseases community are carried out by Task Forces
Yearly State-of-Play

Freely available on www.irdirc.org
“IRDiRC Recognized Resources”

- Label highlighting tools, standards, platforms and guidelines which contribute directly to IRDiRC objectives
- “IRDiRC Recommended” labels given to:
  - Orphanet
  - International Charter of Principles for sharing Bio-Specimens and Data
  - PhenomeCentral
  - ORDO
  - OMIM
  - HPO
  - ICHPT
  - GA4GH Framework for Responsible Sharing
  - DECIPHER
  - TREAT-NMD Patient registries
  - TREAT-NMD Standard operating procedures
  - TREAT-NMD Advisory Committee for Therapeutics
  - Care and Trial Site Registry
3rd IRDiRC Conference

4 plenary session and 3 parallel tracks
- Rare Diseases Research in 2017 – a global view
- Foundational, Diagnostics and Therapeutics Research in 2017
- New Approaches to Rare Diseases
- Trends in the field
- Transforming Rare Diseases Research

www.irdirc-conference.org
IRDiRCs Policies and guidelines

Freely available on www.irdirc.org