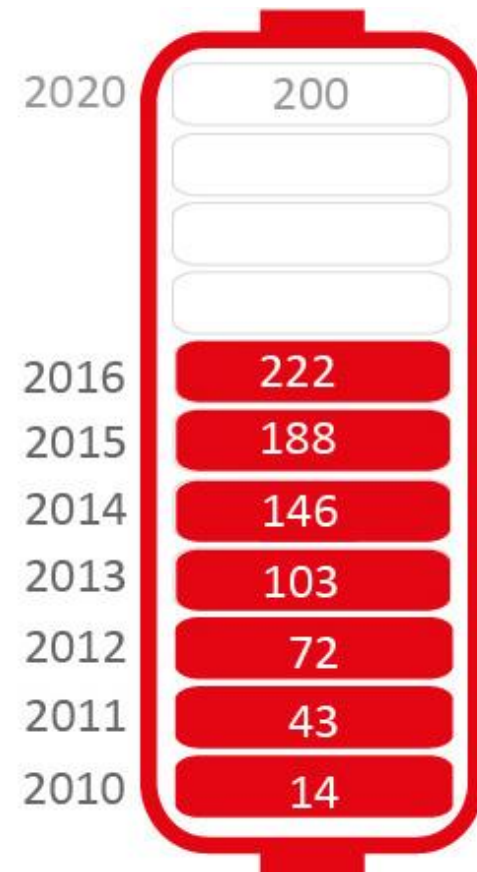




IRDiRCs Current State of Progress-Summary

Number of New Orphan Drugs

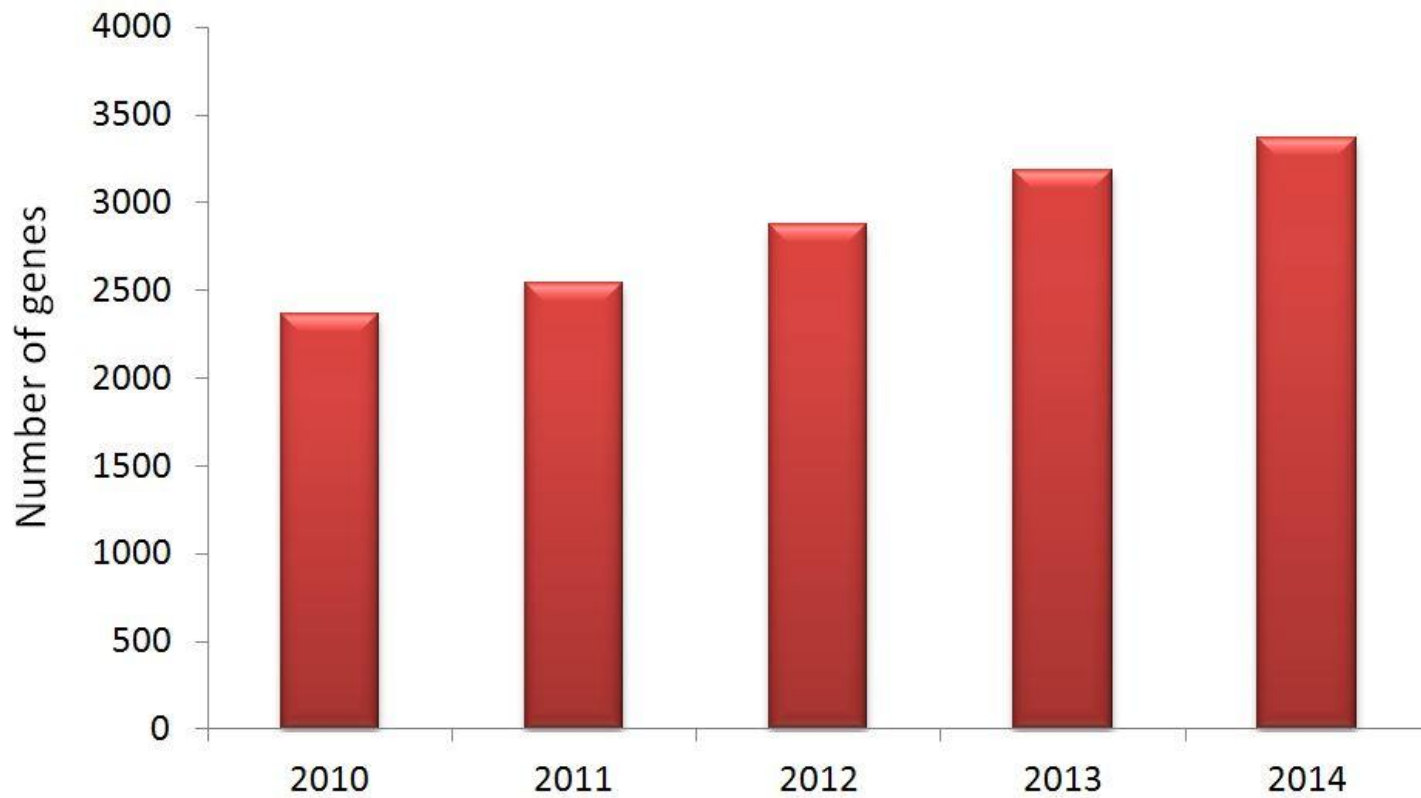
- ▶ Monthly update of number of new indications for orphan diseases
- ▶ Source: EMA and FDA



Updated on www.irdirc.org

Number of Identified Genes Causing Rare Diseases

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



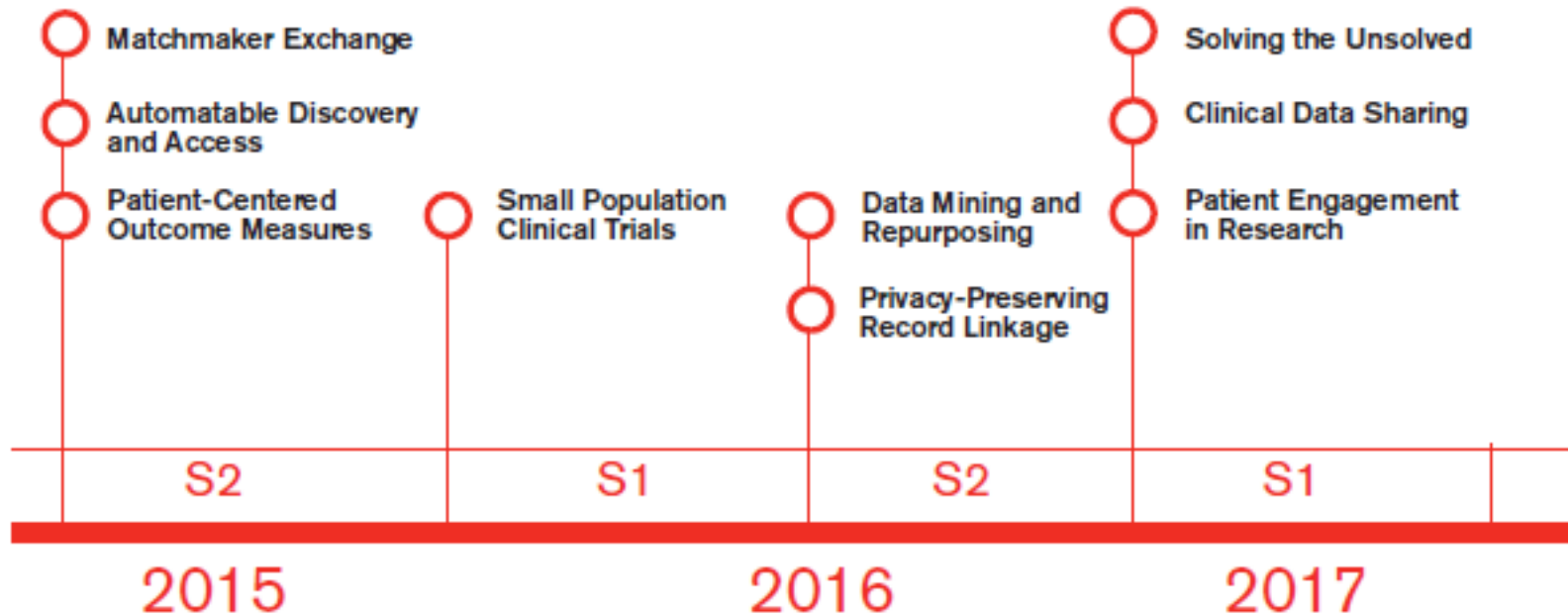
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CONSORTIUM

Source: Orphanet Data

Task Forces in Action

- ▶ Actionables to ensure IRDiRC meets its objectives for the rare diseases community are carried out by Task Forces



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Yearly State-of-Play



“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



▶ www.irdirc-conference.org

- ▶ 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

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IRDiRCs Policies and guidelines



Governance



Freely available on www.irdirc.org