



**Rare Diseases
Ireland**

Rare Diseases Ireland Round Table of Companies

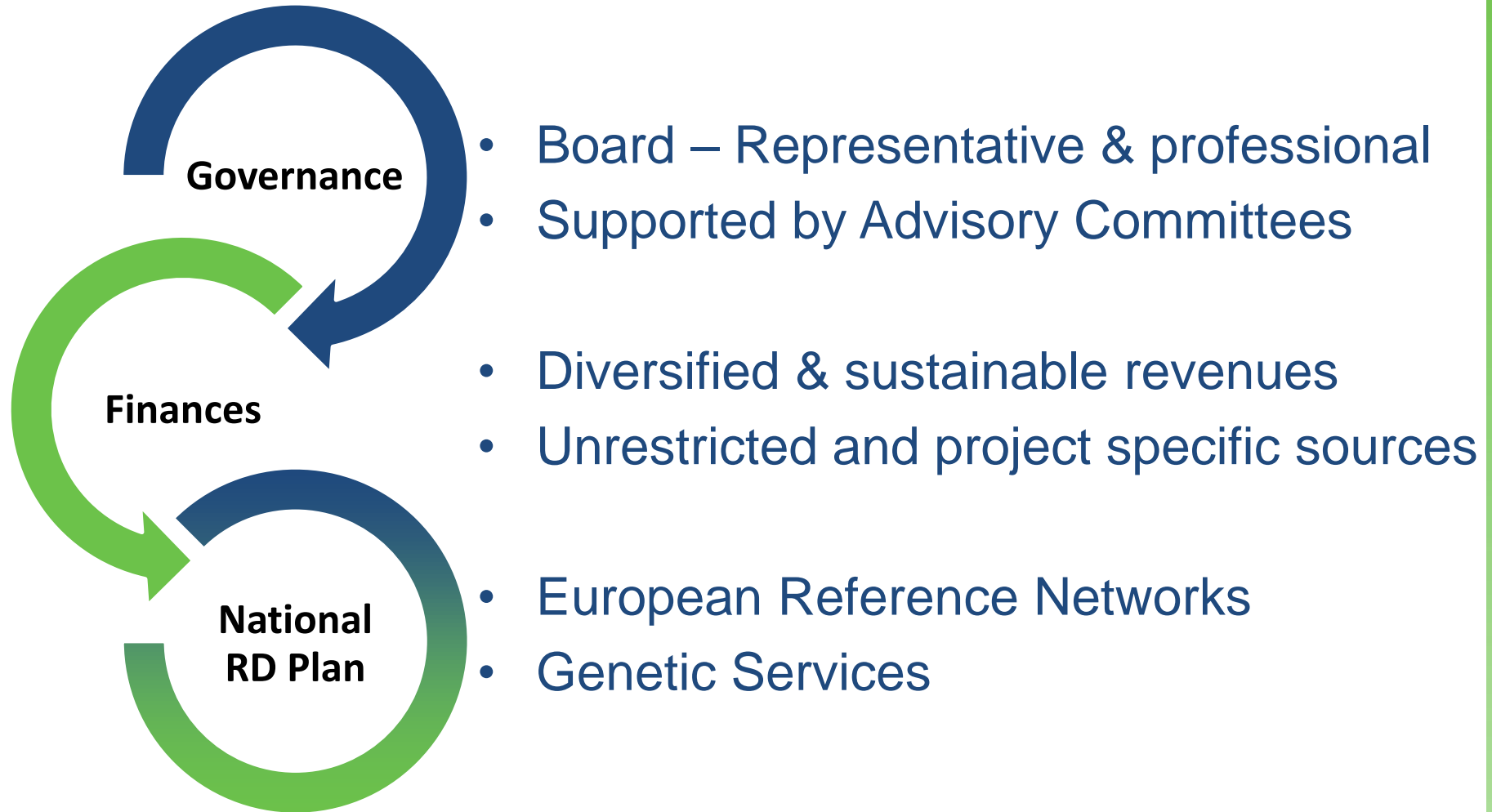
**(RDI – RTC)
22 Nov 2018**

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Interim CEO, Rare Diseases Ireland

Rare Diseases Ireland

- Vision: **better lives and cures for people living with a rare disease**
- Mission: **work across diseases to improve the lives of people in Ireland living with a rare disease. We are committed to the identification, treatment, and cure of rare diseases.**

RDI - Strategy 2019



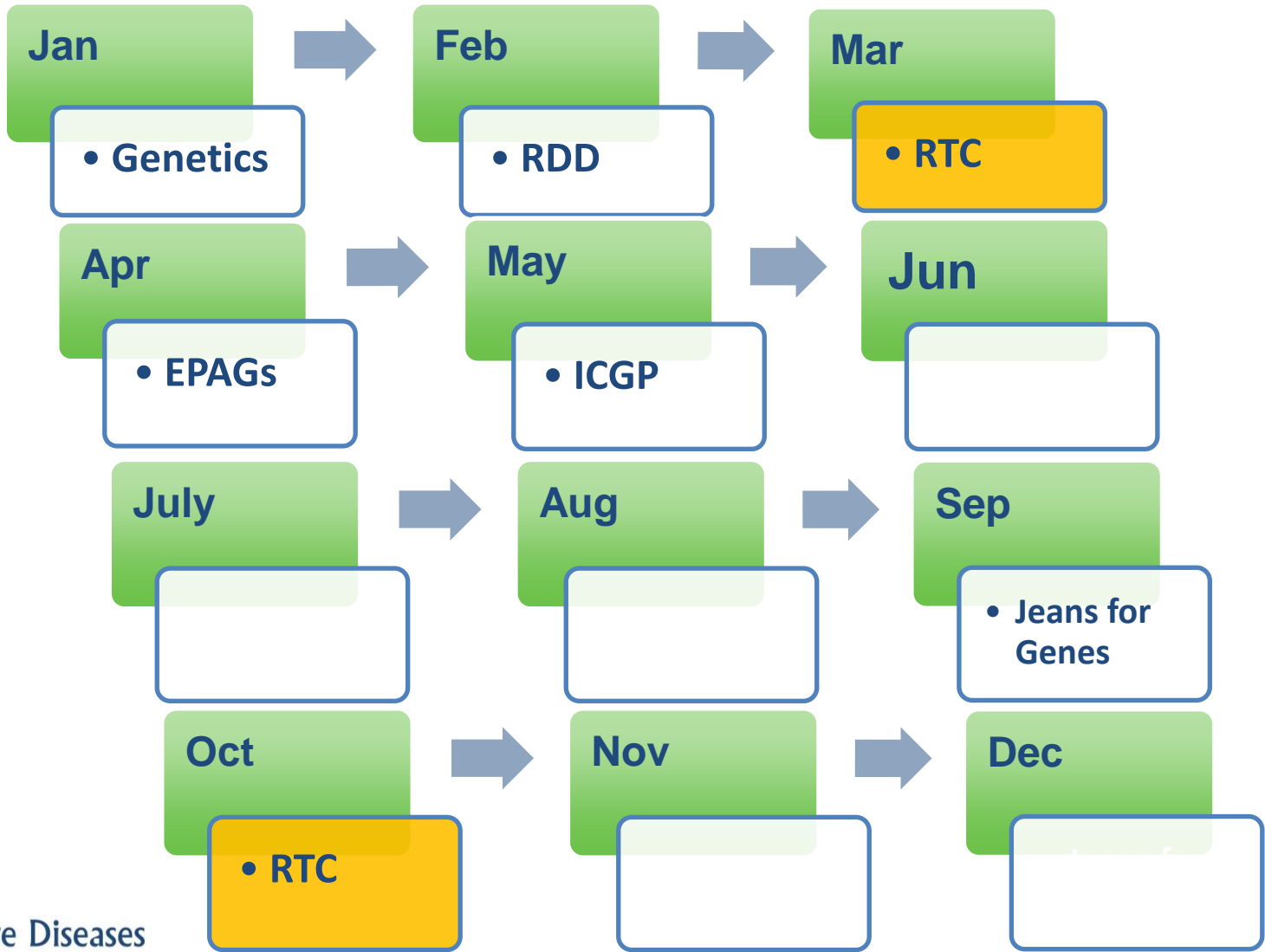
European Reference Networks

- Clinical integration into Ireland
 - 3 ERNs today – 20+ by end 2019
 - Hematology; Skin Diseases; Vascular Diseases
 - Ensure that Irish ‘centres of excellence’ are linked in to ERNs
- Patient organisation integration into Europe
 - 3 EPAGs today – 10+ by end 2019
 - Neurology - PoPSyCLE Foundation, FARA
 - Pulmonary – ILFA
 - Align patient orgs to match ERNs
- Network into GP medical practice

Genetic Services

- Upskilling patient organisations on genetics
 - Change the conversation around genetics
 - Ethics & consent
 - How does diagnosis impact medical practice
 - What are the different types of testing
 - Panel versus whole genome; incidental findings
 - No diagnosis – the diagnostic odyssey
 - Genetic counselling
 - What does a diagnosis mean
 - Who is impacted
- Development/Implementation of HSE strategy
- Training of genetic counsellors

RDI 2019 Calendar of Events



Purpose of RDI-RTC

Establish long-term relationship between RDI and industry, putting patient at the centre of relationship

- Stimulate companies to discover and address patients' needs
- Support companies working with patient groups
- Explore opportunities for collaboration between RDI and industry to meet needs of the patient community
- Promote open and frank interchange of information, in a neutral forum
- Ensure transparency in RDI's relationship with industry

Purpose of RDI-RTC

Provide vital financial support to RDI (via industry membership fees) for unrestricted funding for activities of common interest and benefit:

- Improving access to information
- Raising public awareness
- Empowering patient groups and their advocacy capacity
- Networking patient groups nationally and internationally

Examples of potential activities

- Education:
 - Public: what is genetics?
 - Public: why gene therapies are not to be feared?
 - Industry: Engaging with patients – community advisory boards
- Studies & surveys
 - Impact of living with a rare disease in Ireland

RDI-RTC Rules of engagement

- Rules of engagement
 - Sign up to Code of Conduct
 - Complete RTC membership form
 - Pay membership fees - €3,500/annum
 - NOT an ordinary member of RDI; member only of RDI-RTC
- 2 face-to-face member meetings/annum

RDI-RTC Code of Conduct

- Join to improve understanding of rare disease issues.
- Exclusively discuss issues of common interest to members of RTC.
- Not a platform to advocate or attempt to influence specific decisions or positions of RDI.
- Participation has absolutely no influence on the decisions made by RDI representatives in other settings.
- Respect independence and integrity of RDI, its representatives and members.
- Recognise role of RDI in representing people affected by rare diseases; people who are entitled to express critical views on any relevant matter.
- Recognise role of patient groups in clinical trials and seek to encourage active partnership between patients, health professionals and industry.
- Respect and listen to the other members of the Round Table of Companies.

Rare Diseases Ireland

***NOTHING ABOUT US
WITHOUT US***