

**Perspectives of the International Rare Disease
Research Consortium on the
COVID-19 pandemic**

**Survey Report
December 2020**

IRDIRC



To quote this document

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Preface

We report the outcome of a survey launched by the International Rare Disease Research Consortium (IRDiRC) among its members and scientific experts, providing a unique combined perspective of the three major stakeholders in the research efforts to tackle rare diseases globally. The goal of the survey was to gauge the impact of COVID-19 on the RD research community and to understand how IRDiRC can react to such changes.

1. Is the crisis going to hinder or delay achievement of IRDiRC goals?
2. Are there new and unforeseen issues that have arisen that IRDiRC could address through dedicated actions (task forces, working groups)?
3. What requests or needs by the RD research community IRDiRC may convey to international authorities or bodies?

IRDiRC Vision

Enable all people living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention ¹

Goal 1: Diagnosis



To provide patients with a diagnosis within one year of coming to medical attention or ensure that undiagnosable individuals enter globally coordinated diagnostic and research pipeline

Goal 2: Therapies

To approve 1000 new therapies for rare diseases, with special attention to diseases without approved options



Goal 3: Impact

To develop methodologies to assess the impact of diagnoses and therapies on rare disease patients



Key outcomes

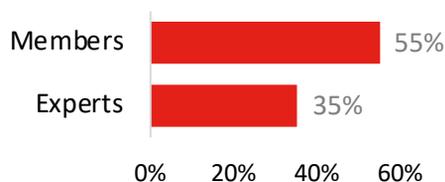
- 1** The impact of rare diseases is real. Rare Disease patients are the most affected ones. The perceived societal impact was high, affecting patients as well as caregivers and families.
- 2** Research and development activities on RD were reported as hampered, delayed, or reduced. Grant-making and managing activities were slowed down or hindered for most funders.
- 3** A slowdown in the progress towards IRDiRC goals was forecasted.
- 4** IRDiRC should stay focused on rare diseases and the set goals. We will maintain the planned activities and roadmap.
- 5** IRDiRC to continue monitoring needs and gaps caused by the pandemic, and to keep gathering information on the developing needs from IRDiRC members and scientific experts.
- 6** IRDiRC should extract lessons learned on this pandemic, from both RD and potentially other disease areas. IRDiRC should use lessons learnt to prepare for the next crisis (pandemic or other).
- 7** IRDiRC strongly recommends that private and public funding bodies maintain investment on RD research and to stimulate research stemming from advancements spurred by the COVID-19 pandemic.

Conducted

18 June - 4 August 2020.

Responses

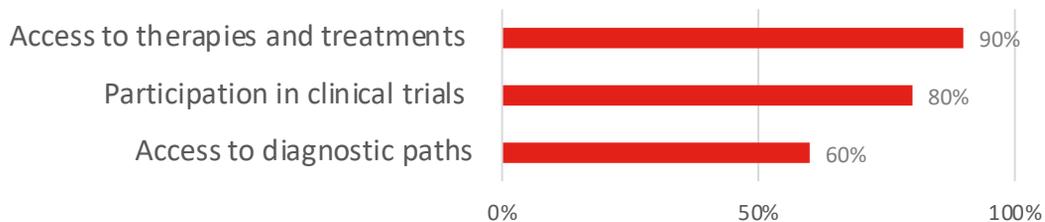
44 out of 95 members and experts
(46% response rate)



Impact on clinical trials and access to treatments

Rare disease patients experienced issues in accessing diagnostic paths and therapies due to the pandemic. Both Patient Advocacy Constituent Committee and Company Constituent Committee perceived impact on rare diseases clinical trials.

Have you, as a Patient Advocacy Organisation, become aware of specific problems experienced by RD patients caused by the pandemic that are hindering accomplishment of IRDiRC goals?



In particular, companies reported impact on their usual R&D processes, highlighting issues in the initiation of new trials and review/approval process. Management of ongoing clinical trials focused on rare diseases was hampered with availability of on-site monitoring visits and patient enrollment/retention. Similarly, rare disease Patient Advocacy Organisations were aware of delays in the starting of trials, or postponement of trials.

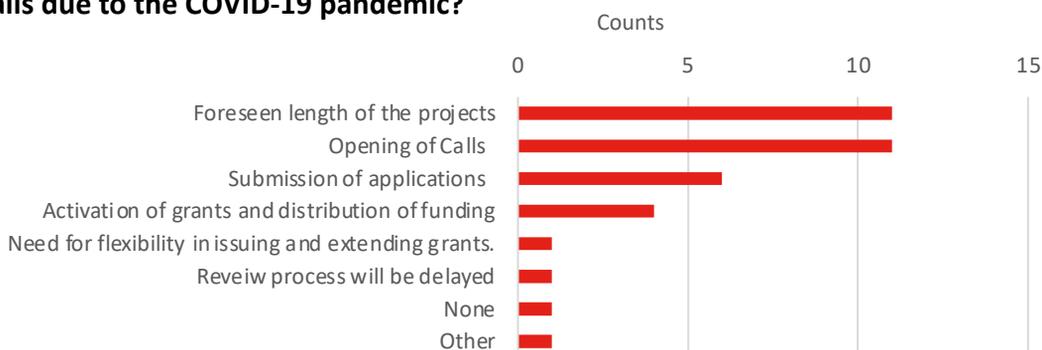
A number of alternative strategies were adapted by companies for ongoing clinical trials such as remote monitoring and remote enrollment.



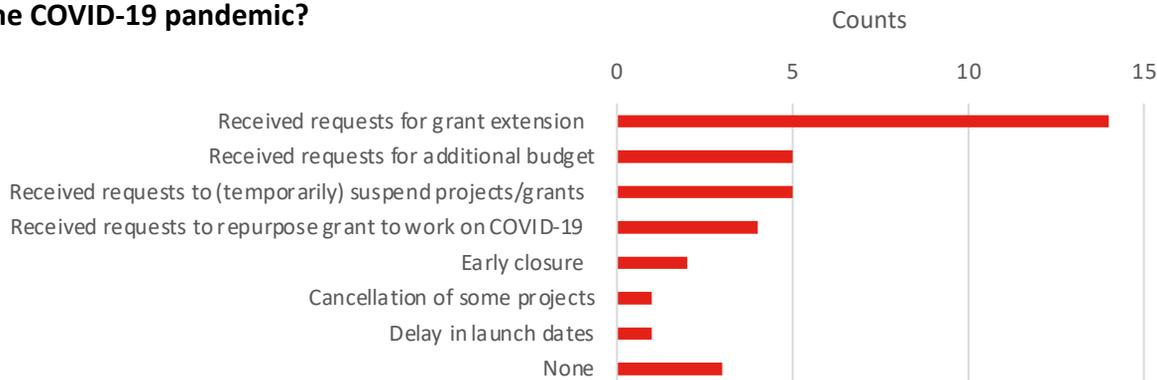
Delays in granting process on rare disease research

Several IRDiRC Funders Constituent Committee members cited delays in the grant-making and grant-managing activities. Many funders received requests to extend project deadlines and have implemented measures to counter the impact of pandemic.

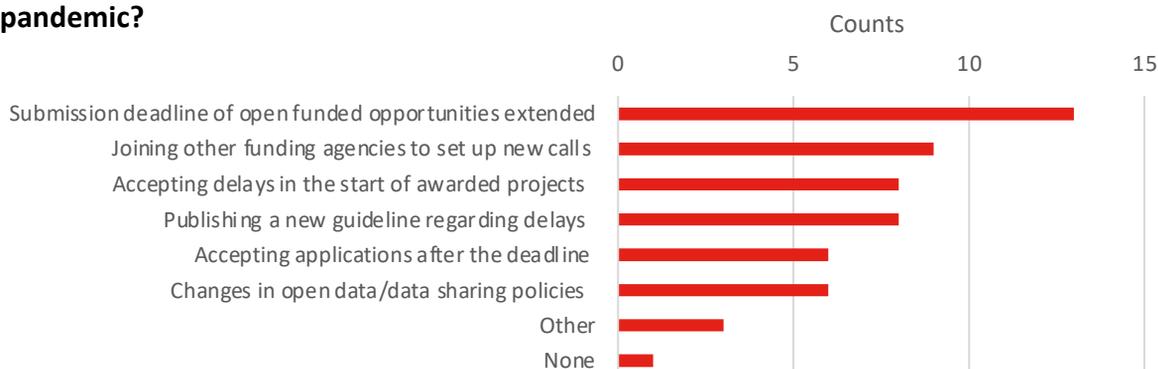
Did you experience any impact on any steps of your usual granting process for rare diseases calls due to the COVID-19 pandemic?



Did you experience any of the following in managing existing rare disease grants due to the COVID-19 pandemic?



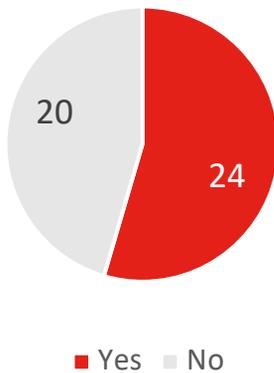
What were the measures/strategies put in place by your organization in response to the pandemic?



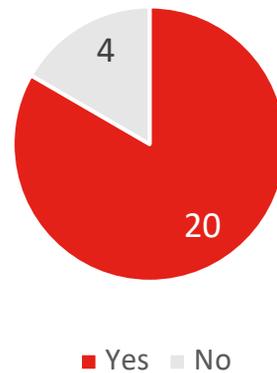
IRDiRC community responded to the pandemic

In response to the pandemic, several IRDiRC member organization have launched their own surveys to understand and map the needs of their communities and stakeholders and are willing to share results with the IRDiRC community.

Have you or your organization launched a survey or collected information related to the COVID-19 pandemic from across your stakeholders?

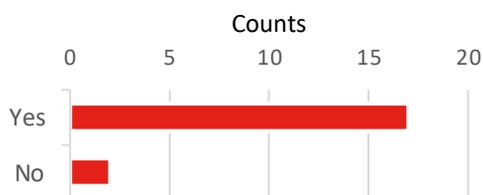


Would you be willing to share key findings relevant to an envisaged impact on the achievement of IRDiRC goals by 2027?



IRDiRC funders supporting COVID-19 research

Did your organization decide to open a COVID-19 related call?

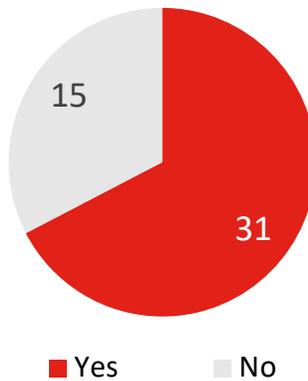


Most of IRDiRC Funders directed some funding efforts on COVID-19.

On track to achieve IRDiRC Goals by 2027

COVID-19 may slow down achievement of IRDiRC goals, but most members and experts believe there is time enough to catch up before 2027. Rare Disease research may benefit from COVID-19 positive spillover effects in biomedical research, for instance faster clinical trials and drug development processes, improved telemedicine and digital tools, and enhanced scientific collaborations in general.

Do you think IRDiRC Goals can be achieved on time by 2027, despite the COVID-19 global outbreak?



“

There is considerable momentum in the rare disease community, especially for therapeutics and gene therapies. COVID-19 will slow us down, but things should get back on track thereafter.

— ANSWERED FROM THE SURVEY

“

SARS-CoV-2 outbreak has seriously impacted ongoing research and clinical trials in rare diseases, as well as re-routed the priority and focus of researchers, companies, funders, and regulators toward fighting CoVID-19 disease.

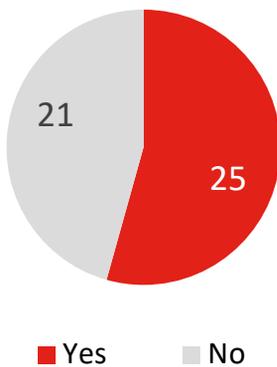
— ANSWERED FROM THE SURVEY



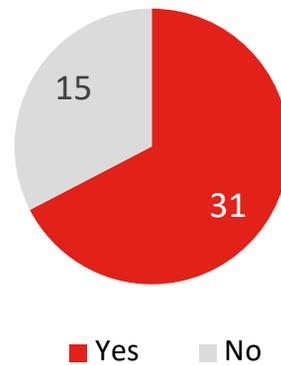
Maintain focus on rare disease research

IRDIRC will advance on the goals set for 2027, according to the roadmap established yearly by the consortium, while monitoring relevant issues and elaborating on needs and opportunities within the activities of its constituencies, scientific committees, task forces and working groups.

Do you see a need for a dedicated COVID-19 IRDiRC Task Force or Working Group?



Would you like to see IRDiRC engaging in an action dedicated to the COVID-19?



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This is not the first, nor will it be the last, major disruption in medical care for people with rare diseases, so lessons learned here might be applied in the future.

— ANSWERED FROM THE SURVEY

“

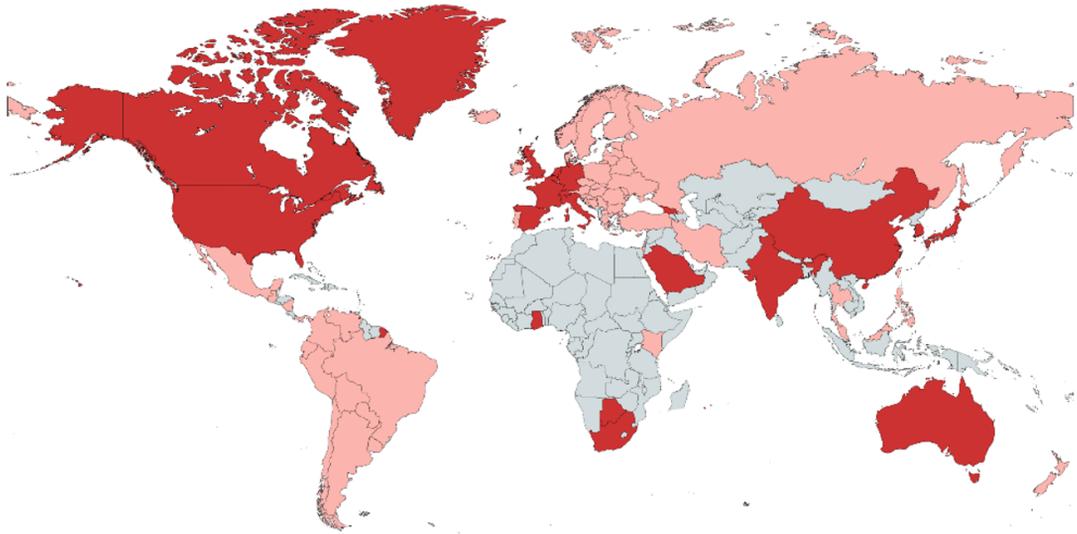
I think this topic should be discussed by each of the committees on a regular basis.

— ANSWERED FROM THE SURVEY

“

I think each funding agency already has many task forces dealing with COVID-19 for their own individual needs and to the extent that rare diseases fit within their portfolios, those needs will be addressed--I don't think an additional IRDiRC task force adds value.

— ANSWERED FROM THE SURVEY



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