

Unmet Needs of Rare Disease Patients during the COVID-19 Pandemic

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Introduction



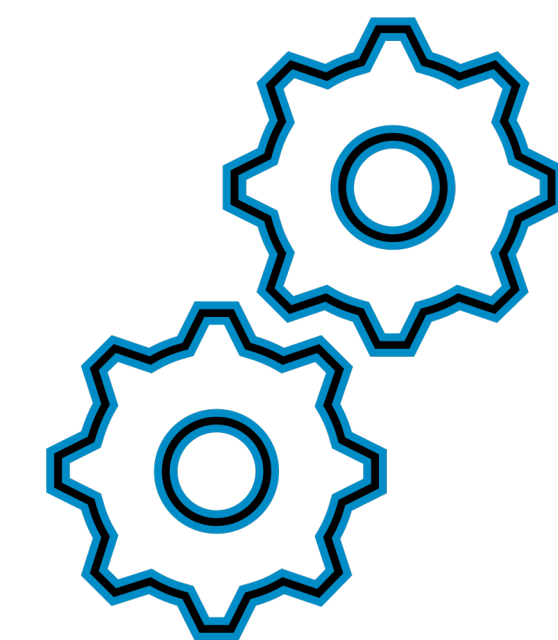
- COVID-19 measures to avoid the spread and to prepare health care system for COVID-19 treatments in hospitals lead to closure of clinics and cancellation of appointments, surgeries, and treatments
- As a consequence, Rare Disease patients as a highly vulnerable group are facing suspension of their healthcare, if any
- Shutdown and defined criteria to leave the house raise concerns and extreme uncertainty amongst Austrian Rare Disease patients and their carers and families

Purpose



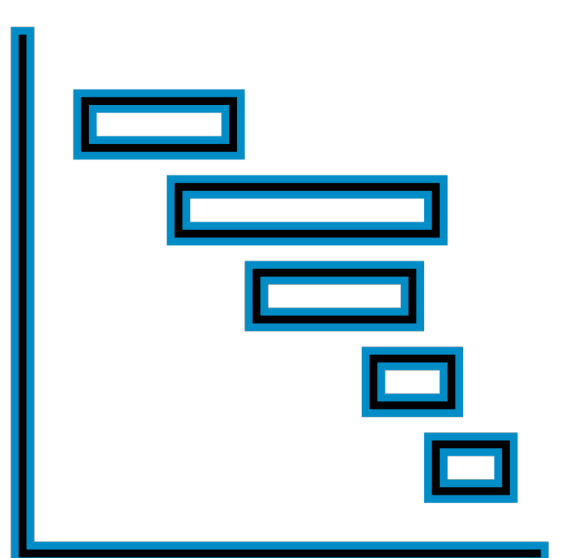
- Outline crisis communication of Austrian Rare Disease Alliance during COVID-19 containment measures and shutdown
- Display concerns and needs of Austrian Rare Disease patients
- Show risks of collateral damage for Rare Disease patients because of barriers to access healthcare
- Provide best practices as potential examples (if any)
- Frame requests to stakeholders with the aim to leave no one behind

Methods



- Joint project Pro Rare Austria and Lobby4Kids
- Conference calls with international Rare Disease and disease specific patient organisations
- Webinar with Pro Rare Austria members
- Pro Rare Austria/Lobby4Kids member survey including health care (medication, appointments, surgeries), screenings, regular assessments, follow-ups, holistic care accompanying children and patients with disabilities, triage, clinical trials, daily life (work, education, schooling)
- Research in literature, online channels and patient forums
- Outreach to supporters for endorsement of statement
- Outreach to media (print and radio)

Results



- Supply issues and shortage of medicines due to repurposing for COVID-19 treatment (e.g. Hydroxychloroquine, Azithromycin)
- Postponement of appointments, screenings, imaging, lab exams leading to delayed diagnosis and intervention
- Suspension of treatments/surgeries increasing the risk of severe deterioration of Rare Diseases (potentially life threatening/ debilitating)
- Interruption of access to holistic care (rehabilitation, physiotherapy, speech therapy,...) reducing its efficacy and sustainability
- Triage guidelines and triage cases reported from abroad raise concerns in Rare Disease patients that access to life saving treatment in case of shortage of hospital resources might be denied
- A certain number of clinical trials for Rare Diseases – in some rare cases the only (experimental) treatment option for Rare Disease patients - are reported to be suspended or halted. Development of orphan drugs and therapies seem to be at risk in the long run
- Accompanying of children and/or people with disabilities reported not to be allowed in some cases
- Lack of flexibility or of definition of work conditions, schooling and childcare are perceived as extremely concerning

Conclusion



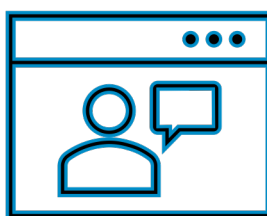
- Risks and collateral damage for the vulnerable group of Rare Disease patients during the COVID-19 pandemic due to interrupted access to healthcare is seen as alerting
- Concerns of Rare Disease patients and patient representatives to be left behind are overwhelming
- Pro Rare Austria's crisis communication with members is seen as reassuring for Austrian Rare Disease patients
- The project team has experienced spirit and momentum despite the uncertain times during COVID-19 pandemic

Requests



- Exclude discrimination in case of triage situation
- Guarantee direct communication between patients and clinicians (enhance telemedicine where useful)
- Benefit from existing infrastructure (staff, labs, imaging devices) to sustain screening and diagnosis
- RD patient involvement in pandemic guideline development
- Ensure accompanying of children/persons with disabilities
- Develop concept to continue clinical trials for Rare Diseases and provide funding for study sites during and after pandemics

References



Sturz D, Promussas I, Kroneis T, Röhl C, 2020. Statement zur Versorgung von Menschen mit seltenen und/oder chronischen Erkrankungen in Zeiten von COVID-19, <https://www.prorare-austria.org/newsroom/aktuelles/news/statement-zur-versorgung-von-menschen-mit-seltenen-undoder-chronischen-erkrankungen-in-zeiten-von-c/>

Sturz D, Promussas I, Kroneis T, Röhl C, 2020. Auswirkungen der COVID-19 Krisensituation für Menschen mit seltenen und/oder chronischen Erkrankungen in Österreich, <https://www.prorare-austria.org/newsroom/aktuelles/news/statement-zur-versorgung-von-menschen-mit-seltenen-undoder-chronischen-erkrankungen-in-zeiten-von-c/>



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