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

Dominique Sturz*, Irene Promussas, Thomas Kroneis, Clas Röhl

1 Pro Rare Austria, 2 Usher Initiative Austria, 3 Lobby4Kids, 4 KEKS Austria, 5 EAT, 6 NF Kinder, ePAG

* presenting authors

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

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)

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)

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 alarming
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