APPLYING LESSONS FROM COVID-19 TO BETTER HEALTHCARE FOR RARE DISEASES
The Canadian Organization for Rare Disorders joins with all Canadians in the fight against COVID-19. But for Canadians with chronic conditions, rare and common, the fight against their disease does not stop. And in many cases, the fight has become even more challenging.

Patients with underlying conditions that compromise the immune, respiratory, and cardiac systems, are more susceptible to COVID-19 and its complications. In early April CORD sent out a survey asking the impact of COVID-19 on rare disease patients and families. Nearly 300 responses were received within days.

Three-quarters of respondents felt they were at “great” or “very great” risk for COVID-19 complications. “My daughter gets every virus and takes longer to recover.” “...repeat serious double lung antibiotic resistant pneumonia history, asthma, auto immune condition, and recent bad flu symptoms further physically weakening.”

More than two-thirds said they were “somewhat” to “very” concerned about seeking healthcare because of fear of exposure to COVID-19. “I’ve been told should I contract COVID-19 I would be low on the list for treatment due to shortage and "survival of the fittest." “Afraid to go to appointments in hospital. Deferring appointments.”

Nearly half of the patients reported difficulty receiving medical care (other than drug treatment), such as delayed or cancelled surgeries, bloodwork, dialysis, and physiotherapy. “The surgery that is not happening would resolve a problem, in the meantime I am not functional and am bedridden and in a lot of pain.” “I need medical follow up because of serious post op complications and about 75% of it is not happening.” “Specialist in a different city.”
Others expressed fear in lack of services if and when needed, “The fear of going into an adrenal crisis during this pandemic.” “I understand the extra caution and hope no flare ups happen during this pandemic....”

Almost half reported difficulty accessing rehabilitation or critical services, including personal support care. “...had to stop all home supports 4x per day due to risk of infection.” “House bound/extreme mobility issues/no one able to help because of mine and their fears/stay at home in effect.”

In terms of drug therapy, nearly 40% said they were experiencing “some” to “great” difficulty getting access to medicines. Some patients said regular medicines (amoxicillin, hydroxyurea) were not available, or they could only get a portion of their regular supply (one month instead of three months) or receive a substitute for their usual medication. “The medication I require is now needed to treat COVID-19 - I won’t get treatment I need because I agree health care professionals do need it first - it’s going to be a long 24+ months.”

Most of the barriers, however, were not attributable to drug shortage but to disruptions due to logistics (computer crashed because of demand, had to call many times, long lines at the drugstore); lack of local supply (had to call four pharmacies, backorder); and safety concerns (products delivered at door, had to drive to a smaller town in safer environment). “Daughter needed a right heart cath[erization] ... to be able to move to a new medication. The heart cath[erization] was cancelled the Wednesday before.” “I was told my prescription may be terminated on April 16 as they are trying to free up Hydroxychloroquine for research into the virus. I was told they [pharmacists] have never heard of my disease.”
Patients also reported notable system delays due to COVID-19, such as the cancellation of a clinical trial for a highly anticipated new medicine, delayed Health Canada approval for a Special Access Program request (no answers), and inability to access bloodwork to qualify for therapy.

Not surprisingly, more than 75% of respondents reported they experienced “some” to “great” anxiety, fear or stress because of COVID-19 and nearly one-half said they had withdrawn emotionally. “I am anxious my lack of follow-up will result in serious problems or that I’ll get COVID and probably die if I go to the hospital.” “very high stress, parent of 4 children, two of whom have specialized needs, panic attacks, deeply struck with grief and anxiety on daily basis.”

What has emerged as some of the “positives” from this crisis? Several patients had access to their healthcare provider through phone or email. Others were able to use telehealth. In some cases, healthcare providers went around the system to provide necessary service. A patient was given referral to an off-site blood lab and arranged follow up with the hematology team by telephone. In another case, a lab worker came to the house to draw blood. Another patient reported that her care team “went ahead and started a life-extending new medicine without the normal pre-requisite heart catherization.”

In a separate survey sent to the pharmaceutical suppliers of rare disease medicines, CORD received assurances that (most) Canadian drug supplies were not in jeopardy. Moreover, they were taking steps to assure procurement, production, and delivery to meet future needs. Some reported making alternative arrangements for drug administration (for example, at specialty pharmacy sites) or supporting home administration. Furthermore, all of the companies responding said that on-going clinical trials would not be interrupted, with alternative arrangements for administration, monitoring and follow-up data collection if necessary.
CORD strongly supports the concerted actions that government and health system are taking in the fight against COVID-19. However, it is equally vital that we do not neglect essential care and treatment for patients with existing health conditions. Unfortunately, the feedback from our rare disease patient survey paints a stark and disconcerting picture of limited, delayed, and denied testing, medical services, surgeries, rehabilitation therapy, supportive care, and medicines, all of which inevitably put patient lives and wellbeing at risk. Equally disconcerting is the apparent lack of education in (some) emergency rooms on practice guidelines for managing COVID-19 patients with certain high-risk pre-existing conditions, including rare disorders, as well as those on certain medications. Our public health experts tell us that this is most likely the first of two or three waves and that the impact of COVID-19 will be felt until 2021. This will have a profound effect on patients with rare diseases.
BIG LEARNINGS

Here are BIG LEARNINGS from the COVID-19 crisis that would improve rare disease patient outcomes and experience, and, as importantly, the experience of the healthcare providers.

1. You gotta have a plan. Every rare disease patient needs to have an individualized plan developed in consultation with the patient and family and readily available (in electronic form), to all providing healthcare services, especially in emergency situations.

2. Rare disease is a distinct medical speciality. Like oncology, cardiology, neurology, endocrinology, and pediatrics, rare disease needs comprehensive specialty centres networked to localized practitioners and community-based services.

3. Deal with the crisis, but don't sacrifice those with other healthcare needs. Set up a team to identify, prioritize, triage, and alternatively resolve the needs of those with chronic and other healthcare conditions.

4. Canada needs an innovative pharmaceutical industry. Companies in Canada are stepping up to assure adequate supplies of emergency drugs and medical devices and are partnering with researchers to develop new tests, therapies, and vaccines for COVID-19. At the same time, they are ensuring that Canadians with rare and common conditions have continued access to the drugs, tests, and other technologies needed. Perhaps this is the opportunity to develop truly effective public-private partnerships.
The problems reported by rare disease patients and families were not directly caused by COVID-19. They are manifestations of pervasive deficiencies and dysfunctions in our healthcare system and beyond. Sadly, it has taken a pandemic to bring these starkly to the surface. It is now our responsibility to expose these, put these before the appropriate authorities and policy makers, and to advocate for changes ... before the next wave or crisis.

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