Putting the COVID-19 vaccines in context for people with rare diseases

For people living with a rare disease, the perception of a health threat due to COVID-19 is high or very high in 71% of cases.[1] As the first vaccines to prevent COVID-19 in Europe have been approved by the European Medicines Agency, people living with a rare disease, alongside all European citizens, are excited to take a step closer to "normality" after an extraordinary year.

Although life is never completely normal when you live with a severe condition, normality is when you can visit your hospital again with no fear of contracting coronavirus. Nonetheless questions remain about vaccines for this vulnerable population.

On 11 December the European Medicines Agency (EMA) organised the first public hearing on the COVID-19 Vaccines development and approval process, following a request from EURORDIS and EPF to set up such a meeting. They have since set up a subsequent hearing as it serves as a valuable opportunity for the public to hear from experts from across the EMA about the work they are doing to prepare Europe for the vaccines, and to hear questions about European citizens' needs.

Francois Houyez, Information & Access to Therapies Director & Health Policy Advisor at EURORDIS-Rare Diseases Europe, spoke at the hearing and raised five crucial points that related, in particular, to the vaccine from the perspective of people living with a rare disease. Watch the full video!

1. **EURORDIS-Rare Diseases Europe trusts the high quality science-driven evaluation vaccines have gone through at the EMA**. From working closely with the agency over the years we have learned, observed and witnessed how they have worked.

2. **We believe that European Union citizens are in a good position to receive a safe and effective vaccine**, due to this process, as well as the probability of having freedom to choose between two, three or more vaccines. Over 1.5 billion doses have already been ordered at a negotiated price that will be reimbursed within the EU.

3. **EURORDIS asks that people living with a chronic and/or rare disease be among the first beneficiaries of the vaccines**, following the elderly and healthcare professionals.

4. **We are getting prepared across the rare disease community**.

   Member organisations have engaged with their European Reference Networks, such as patient advocates for Rett syndrome, where expert clinicians explained the lower risks of experiencing a side effect to a vaccine compared to catching the virus.

   We are also collecting and asking the specific questions linked to the safety of vaccines in certain rare diseases, so we can ensure that patients are as well informed as possible. For example:

[1] EURORDIS Rare Barometer survey on COVID-19, November 2020: https://download2.eurordis.org/rbv/covid19survey/covid_infographics_final.pdf

- **Dravet syndrome:** A fever as a side-effect of the vaccine could trigger seizures. And could there be interactions between vaccines using the mRNA technology and antisense therapies also based on mRNA?
- **Hereditary Haemorrhagic Telangiectasia, rare cancers, immune deficiencies:** Blood transfusions and immuno-suppressants are used to treat these conditions. Is there a need for a wash-out period?
- **Auto-inflammatory diseases**: Will patients need to stop anti-inflammatory treatment? If so, how long before and for how long?

5. **Discussions should be on-going for the continuation of clinical trials for vaccines**, for example cross-over design or active control trials, and for real time surveillance post-authorisation, for example using mobile apps for reporting any feedback.

We encourage patients to express their concerns about the vaccines now, there is still time. For example, if you are in a clinical trial for a treatment for your disease, do ask your doctor if the study protocol authorises the use of that vaccine. EURORDIS is confident the EMA and national authorities will continue to listen and to respond to all questions the public might have: this is an essential component in building trust in the vaccine across Europe.