



European  
Reference  
Networks

# European Reference Networks: COVID-19 vaccination program

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2021

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## Introduction

Various Member States are setting priorities in the vaccination programs, including rare diseases.

There are some rare disease patients for whom vaccination can be contraindicated.

As European Reference Networks (ERNs) are formed by experts in rare diseases, we asked all ERNs to provide their opinion on the priorities and contraindications for patients with a rare disease within their network.

The results were discussed during the internal ERN-Coordinator group meeting of January 27<sup>th</sup>, 2021.





## European Commission, Health and Food Safety

### Board of Member States



### 24 ERN Coordinators

The logo for European Reference Networks, consisting of a circular arrangement of blue dots of varying sizes.

### Patient representatives

The logo for EURORDIS (Rare Diseases Europe), featuring stylized human figures in blue and pink, surrounded by colorful stars, with the text "EURORDIS RARE DISEASES EUROPE" below.

## Background

- We advise that the general recommendations of the EMA regarding new medicines need to be followed.
- Our recommendations will not be complete, and apply to the **adult** population.
- Our recommendations are based on the expert opinion as evidence is lacking.
- Evidence needs to be gathered regarding rare disease patients in the vaccination program, even better will be to set up clinical trials.



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## Notification

Our expert opinion-based recommendations for adults only, can be no more than a start with the ultimate goal to have a complete overview of recommendations within the years to come,

Our recommendations will need very regular updates.

Up to date positions will be available on the websites of many ERNs.



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## Rare diseases with priorities for COVID-19 vaccination

- Related to Respiratory problems
- Related to Cardiovascular problems
- Related to Immunodeficiency or Cancer
- Related to Endocrine diseases
- Other Rare Disease patients  
e.g. Blindness, Epilepsia, Metabolic diseases, etc.



## Related to Respiratory problems

- Rare disease of the respiratory system, such as alpha-1-antitrypsin-deficiency, pulmonary hypertension, Interstitial lung diseases.
- Upper airway obstructions that require respiratory support (CPAP, BiPAP, tracheostoma etc): specifically, Apert, Crouzon and Pfeiffer syndrome, facial dysostosis (e.g. Treacher Collins, Nager and Miller syndrome), Robin sequence, laryngeal and tracheal cleft and stenosis.
- Weak respiratory muscles and/or deformities of the chest wall or upper airways.



## Related to Cardiovascular problems

- Primary arrhythmia syndrome and a history of symptomatic arrhythmias.
- Cardiomyopathy (or congenital heart disease) and risk factors (reduced left ventricular systolic function, heart failure, NYHA III/IV or pulmonary hypertension).
- Carriers of a (probable) pathogenic variant in the cardiac sodium channel gene SCN5A.
- Collagenopathies which cause cardiovascular complications.



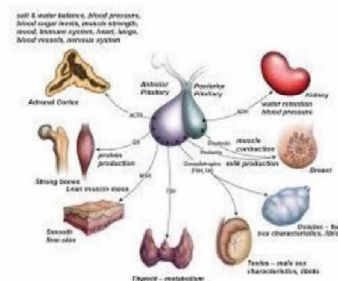
## Related to Immunodeficiency or Cancer

- Rare cancer within 2 years after diagnosis.
- Treated haematological malignancies.
- Immunosuppressive therapy and advanced chronic kidney disease.
- Solid organ transplantation or chronic allograft dysfunction.
- Disorders of calcium and phosphate complicated with hypocalcaemia and/or chronic renal disease and/or immune deficiency.
- APECED/APS1 or auto-immune poly endocrinopathy type 1.
- Auto-immune blistering diseases (AIBD) as they use high dosages of corticosteroids and immunosuppressive drugs, which highly increase risk and severity of infection by COVID-19.
- Chronic haemolytic anaemia with splenectomy.



## Related to Endocrine diseases

- Primary hyperaldosteronism.
- Cushing's syndrome: comorbidities (hypertension, diabetes mellitus) with shown increased risk for severe course of COVID-19.



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## Other Rare Disease patients

- Rare Eye Diseases, as they cannot apply barrier gestures.
- Advanced liver cirrhosis.
- Amino and organic acids-related disorders (AOA).
- Disorder of pyruvate metabolism, Krebs cycle defects, mitochondrial oxidative phosphorylation disorders, disorders of thiamine transport and metabolism (PM-MD).
- Carbohydrate, fatty acid oxidation and ketone bodies disorders (C-FAO).
- Lysosomal storage disorders (LSD).
- Haemoglobin disorders as sickle cell disease or thalassaemia major / intermedia with comorbidities.
- Systemic forms of epidermolysis bullosa and skin fragility diseases.
- Patients with cutaneous Auto immune Bullous diseases
- Women with incontinentia pigmenti
- Difficult-to-treat epilepsies

## Others related to rare disease patients

- Parents or guardians of children undergoing intensive chemotherapy or stem cell transplantation.
- Parents or guardians of new-borns with a congenital malformation.
- Parents or guardians of children with autoimmune disorders receiving intensive immunosuppression.
- Family members of children with immunodeficiencies.
- Parents or guardians of children who are hospitalised for a long period of time.



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## Contra-indications for COVID-19 vaccination

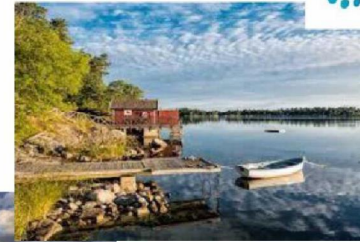


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- Patients with rare neuromuscular diseases who may have an option with AAV9 treatments should avoid AAV virus mediated vaccines.
- In general caution should be taken with patients involved in gene therapy trials.
- Some patients participating in clinical trials may have contraindications dependent on the kind of clinical trial.

Please note that COVID-19 vaccines neither are tested for the paediatric age group nor are vaccinations recommended in the paediatric age group undergoing intensive chemotherapy or allogeneic haematopoietic stem cell transplantation for reasons of lacking or incomplete immune response.

Different experiences,  
resources  
and problem solving  
attitudes



Although we realise that our list is incomplete,  
we do wish that it can be of help.

