



The role of hospital pharmacists in gene therapy preparation

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WHAT WAS DONE?

- Voretigene neparvovec: a gene therapy product indicated for treatment of patients with vision loss due to inherited retinal dystrophy caused by biallelic RPE65 mutations.
- Hospital pharmacists, as part of multidisciplinary team, play important role in preparation of gene therapy product voretigene neparvovec (Luxturna®) at the University Eye Clinic, University Hospital Sveti Duh, Zagreb, Croatia.

WHY WAS DONE?

- The gene therapy product can be distributed only through treatment centers where qualified staff have participated in the mandatory Risk management plan (RMP) education program required by EMA.
- Since voretigene neparvovec has to be transported and stored frozen at $\leq 65^{\circ}\text{C}$, has short shelf life after dilution (4 hours), contains genetically modified organisms and must be handled according to local biosafety guidelines, there was a need for establishing standard operating procedures (SOPs) for each step of the treatment process.

HOW IT WAS DONE?

MULTIDISCIPLINARY TEAM

- Pediatric ophthalmologist
- Inherited retinal disease specialist
- Retinal surgeons
- Pharmacists
- Nurses

STANDARD OPERATING PROCEDURES

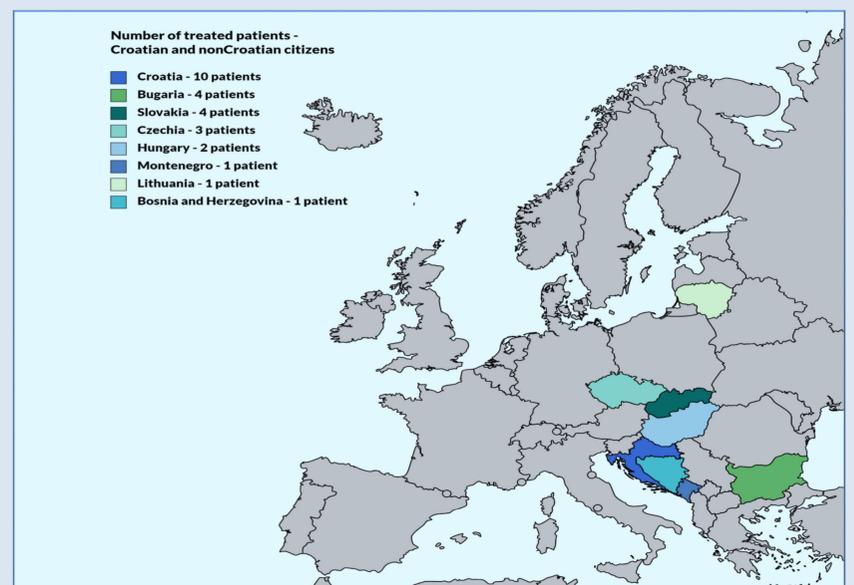
- Ordering process
- Storage of the product
- Coordination between members of the multidisciplinary team
- Preparation of the product
- Disposal of waste

ASEPTIC PREPARATION

- Aseptic conditions in Class II vertical laminar flow biological safety cabinet (BSC)
- Preparation in accordance to pharmacy manual which is ensured by the manufacturer

WHAT WAS ACHIEVED?

- Prevalence of inherited retinal dystrophy associated with biallelic RPE65 mutation is 1:200 000.
- Croatia (population of 3,8 million): approximately 19 individuals with biallelic RPE65 mutation.
- 13 of them were detected since 2020.
- Croatian and non-Croatian citizens were treated (total number of patients 26).



- There were no registered side effects which could be associated with errors during the preparation.

26 patients

Croatian and non-Croatian citizens

Age 6-66

47 eyes

Viable retinal cells left are required

Some patients have viable retinal cells in just one eye

94 single dose preparation

For each administration two single dose syringes are required

One syringe for administration and one „backup“

WHAT IS NEXT?

- Increasing number of gene and cell-based therapies.
- The need for continuous education of hospital pharmacist and exchange of their experiences is greater than ever.

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