

● 28.11.2023.

# IV REGIONAL CONFERENCE ON RARE DISEASES

**THEME: GENE THERAPIES IN RARE DISEASES**

HOTEL SHERATON , POLGAR ANDRAŠA 1, NOVI SAD, SRBIJA 21000 - HIBRIDNA KONFERENCIJA

09:00 – 9:30 Registration & welcome coffee

9:30 – 10:00 *Greetings:*

- **Bojana Miroslavljević**, Association "Life", Rare Diseases Database for Balkan
- **Jelena Janković**, Ministry of Health, Serbia
- **Prof. dr. Sanja Radojević Škodrić**, National Health Insurance Fund, Serbia
- **Mrs. Tamara Vučić**, First Lady of Serbia

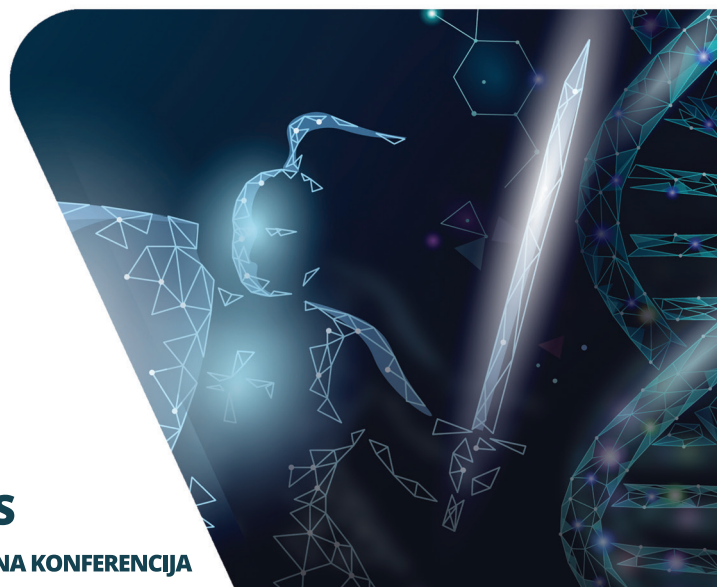
## Introduction - Gene Therapies Regulation

- 10:00 – 10:15 • *Presentation of amendments to the Rulebook on the method of drug control initiated by C4IR*  
**Jelena Bojović, Branislava Gemović** PhD, Centre for the Fourth Industrial Revolution C4IR
- 10:15 – 10:30 • *Digitization: valuable partner in fighting rare diseases*  
**Nevena Veljković**, Heliant LLC & science advisor - Vinca Institute of Nuclear sciences, University of Belgrade, National Institute of the Republic of Serbia
- 10:30 - 10:45 • *The role of The Medicines and Medical Devices Agency of Serbia (ALIMS) in the availability of genetic therapy in Serbia*, **Jadranka Mirković**, Medicines and Medical Devices Agency of Serbia (ALIMS)
- 10:45 - 11:00 • *Ethics and the patients perspective in gene therapies*  
**Goran Vasović**, Ethics Committee of Serbia
- 11:00 - 11:15 • *Challenges and opportunities with access to gene therapies in Europe*  
**Elisabetta Zanon**, Director, EU Public Affairs & Advocacy, Alliance for Regenerative Medicine
- 11:15 - 11:30 • *Access to genetic therapy – when is genetic therapy the best choice for a patient?*  
Assistant Prof. Dr. **Predrag Rodić**, University Children's Clinic Belgrade

11:30 - 11:45 Coffee break

## Gene Therapies - Overview

- 11:45 – 12:00 • *Genetic therapies: from technology to clinical studies*  
**Maja Stojiljković**, PhD, Full Research Professor Institute of Molecular Genetics and Genetic Engineering, University of Belgrade
- 12:00 – 12:15 • *Genetically designed therapies - example of SMA*  
**Miloš Brkušanić**, PhD, Research Associate, University of Belgrade - Faculty of Biology
- 12:15 - 12:30 • *Orphan designations granted to gene therapy in Europe: analysis and evolution over the last two decades*  
**Melania Cruciani**, Orphanet, Clinical Trials and Orphan Drugs Database Manager, France



## Gene Therapies - Overview

- 12:30 - 12:45 • **Intrapataminal gene therapy for AADC deficiency – approach and results**  
**Prof. Thomas Opladen**, MD, MHBA Division of Pediatric Neurology and Metabolic Medicine, Heidelberg University Hospital, Germany
- 12:45 - 13:00 • **Cross border treatment : three years of real life experience**  
**Prof. Mladen Bušić**, PhD, University Hospital "Sveti Duh" Zagreb, Croatia
- 13:00 - 13:15 • **From genotype to phenotype and treatment od blindness**  
**Ass. Prof. Mirjana Bjeloš**, PhD, University Hospital "Sveti Duh" Zagreb, Croatia
- 13:15 - 13:30 • **The bridge between biomedical research on advanced treatments and its transfer to patients: an academic facility**  
**Mojca Benčina**, Center of excellence for the Technologies of Gene and Cell Therapy from the National Institute of Chemistry, Slovenia
- 13:30 - 14:30 **Lunch**
- 14:30 – 14:45 • **Bringing a gene therapy to patients: the first experiences of a company**  
**Thomas Bols**, PTC Therapeutics, Head of Government Affairs and Patient Engagement, EMEA, France
- 14:45 – 15:00 • **The role of patient advocacy in supporting gene therapy**  
**Vivian Fernandez**, RegenexBio, USA
- 15:00 - 15:15 • **Empowering Patients: Gene Therapy and Patient Engagement in Rare Disease**  
**Kendall Davis**, ICON - Patient Advocacy Strategy, director
- 15:15 - 15:30 • **What patient groups can do to prepare their communities for gene therapy**  
**Philipp Von Gallwitz**, Patient driven solutions, ADMEDICUM

## Panel discussion: Genetic therapies from different perspectives

Moderator: **Ivana Badnjarević**

- 16:00 – 17:00 • **Challenges in the availability of gene therapy in the Pharma Industry**  
**Davor Duboka**, PTC Therapeutics, Associate Director, Patient Advocacy, France
- **Zoran Aćimović**, CRA Academy
- **SMA patient perspective Olivera Jovović**, NORBS The National Organization for Rare Diseases of Serbia
- **Epidermolysis bullosa EB - Patient perspective Sandra Pavlović**, Debra Serbia
- **Parental experience Sonja Todorović**, mother of SMA boy Lav

## 17:00 – 17:15 Q&A

## 17:00 – 17:15 Conclusions and closing

### Projekat podržali:



Република Србија  
Министарство за рад, запошљавање,  
борачка и социјална питања

**#СРБИЈАБЕЗБАРИЈЕРА**

Реализацију програма подржао је Сектор за заштиту особа  
са инвалидитетом Министарства за рад, запошљавање,  
борачка и социјална питања

### Projekat pomogli:

