

## “Emerging therapeutic agents and therapies”

### Contents:

- Introduction: traditional medicines like small molecule inhibitors (for more than 100 years) and antibody-based therapies (for the last 30 years) (both targeting protein);
- Short introduction to molecular biology (DNA-RNA-splicing-protein);
- New emerging drugs and therapies: nucleic-acid therapeutics:
- antisense oligos (ASO) (targeting RNA);
- miRNA, RNAi, siRNA-based drugs (targeting RNA);
- Aptamers (DNA, RNA, protein);
- Genome editing (or genome engineering): meganucleases; Zn finger nucleases; TALENS; CRISPR/Cas9; gene therapy; delivery problem in gene therapy; ethical issues of gene therapy;
- Other RNA-based therapies or RNA for therapeutics (mRNA therapy; modified snRNAs (ExSpeU1); trans-splicing (SMART));
- Biologics or protein therapeutics (human antibodies-monoclonal antibody therapy);
- Intracellular delivery problem for new emerging drugs – if **Nanotechnology** can help?; Spherical nucleic acids (SNA) as delivery for ASOs and RNAi therapy;
- useful links, sites, pages, etc.

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1989 graduated Vilnius University, faculty of Chemistry

2003 PhD in Medical Virology at Uppsala University, Sweden

2004-2009 PostDoc at Aarhus University, Denmark and Braunschweig Technical University, Germany

2009 and up to date – PostDoc at Max Delbrück Center for Molecular Medicine, Berlin, Germany

Scientific interests: RNA biology, RNA processing, splicing, RNA therapy