GET+VVIRAL: a Purification Toolbox for the Gene-Editing Tools and Viral Vectors

Stefano Menegatti
North Carolina State University, North Carolina, United States

Gene Editing Products (GEPs) are poised to become the focus of next-generation treatment of severe cardiovascular, muscular, metabolic, neurological, hematological, and ophthalmological disorders, infectious diseases, and cancer. In this context, CRISPR nucleases (e.g., Cas3, Cas9, and Cas13) and viral vectors (e.g., adenovirus, adeno-associated virus, and lentivirus) are playing a growing role owing to their superior targeting and therapeutic efficacy. The research on increasing the potency and safety of GEPs is progressing at outstanding pace, with 100s new products accessing the clinical pipeline every year. Along with medical uses, GEPs are successfully applied in numerous biotech fields, including the development of new biomaterials, plants, and livestock. As a result, the GEP market is undergoing an explosive growth, with a revenue generation estimated at $20B by 2030, while GEPs are projected to become mainstream therapeutics by 2050, impacting 60 - 80 million patients per year worldwide. With such explosive growth, GEPs are posing critical questions to the biomanufacturing industry: how to produce affordable, high-quality nucleases in sufficient amount to supply the global biotech and biopharma demand? How to manufacture disease-/patient-specific viral vectors affordably? When is continuous manufacturing of GEPs needed and how can it be achieved? In response to these challenges, our team has introduced a portfolio of purification tools for next-generation biomanufacturing of GEPs. In this talk, I will present our current efforts: (1) LigaGuardTM adsorbents for continuous purification of protein and virus therapeutics by capturing process-related and product-related impurities via \"flow-through affinity chromatography\"; (2) SMART technology for the purification of CRISPR ribonucleoproteins, namely Cas nucleases primed with the desired guide RNA; and (3) adsorbents for the serotype/pseudotype-agnostic purification of AAVs, AdVs, and LVVs.