Precision NanoSystems
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PRECISION NANOSYSTEMS
Create Transformative Medicines™

Technologies and solutions for advancing RNA, small-molecule and protein nanomedicine

Precision NanoSystems empowers researchers to develop genetic medicines through its NanoAssmblr technology, helping companies solve drug delivery challenges in both cancer and infectious diseases.

Precision NanoSystems Inc. (PNI), based in Vancouver, Canada, provides end-to-end solutions for companies developing medicines in oncology, infectious diseases and rare diseases. Drug delivery technologies designed to protect and control the biodistribution of the active biologic or small molecule are driving innovations in these areas. PNI's proprietary technologies include its NanoAssemblr microfluidic-based continuous flow manufacturing platform for the development and manufacture of nanoparticle drug delivery systems, and its lipid nanoparticle delivery technology for RNA medicines. PNI also establishes strategic partnerships with technology providers in bio-processing to deliver solutions.

The NanoAssemblr Blaze system.

Transformative medicines: the opportunity

After small molecules and protein biologics, gene therapy and RNA medicine represent the next model of drug innovation. For example, RNA vaccines against cancer and infectious diseases, are a promising technology as they are faster and easier to develop and manufacture than conventional vaccines. This is because RNA vaccines are completely synthetic and do not require cell culture. Instead, it is the genetic code that is delivered, enabling the recipient's cells to produce representative but harmless parts of viral antigens or cancer mutations. The speed advantage of RNA has spotlighted the technology in the current pandemic response. With its technologies and services, PNI supports many COVID-19 vaccine developers to overcome the challenges inherent in emerging medicines. "We continue to build global partnerships to further lower these barriers," said James Taylor, PNI's CEO.

PNI recently received a commitment of up to $18.2 million in support from the Government of Canada under the Innovation, Science and Economic Development's (ISED) Strategic Innovation Fund (SIF) to develop a COVID-19 vaccine. PNI will use the investment to advance a best-in-class COVID-19 mRNA vaccine candidate to clinical trials.

Challenges and solutions

A challenge for RNA medicine is getting the RNA into cells where it can be biologically active. PNI's lipid nanoparticles (LNPs) protect the RNA payload and release it into the interior of the target cell through receptor-mediated endocytosis. PNI's proprietary library of LNP formulations designed for vaccine applications employ cationic ionizable lipids that facilitate RNA encapsulation and release. These LNP formulations can be licensed for development and clinical use.

For clinical approval, LNPs must be of consistent size and morphology as heterogeneity could mean that the product accumulates throughout the body instead of targeting one area. Consistency is also important for the tangential flow filtration and sterile filtration steps of processing, to avoid clogging the filters. PNI's microfluidic technology produces LNPs under more controlled conditions than conventional mixing. In-house and peer-reviewed studies[1] have confirmed consistent size, polydispersity, payload encapsulation and biological activity within and between batches produced using the same equipment. The NanoAssemblr platform, including its good manufacturing practice (GMP) system, uses PNI's NxGen microfluidic technology driven by validated software to control the nanomedicine self-assembly process both spatially and temporally to produce a uniform, high-quality nanomedicine drug product.

PNI supports its clients with custom research projects through its Formulation Solutions service and clinical implementation through Clinical Solutions. The former can involve proof-of-concept and formulation development projects. The latter involves scale-up and process development, preparation of batches for GLP-tox studies in a pre-GMP environment and technology transfer to a GMP facility for manufacturing clinical or commercial scale batches, with PNI providing on-site training, support for the preparation of product-specific GMP master batch records and chemistry manufacturing and controls.

Partnering to accelerate transformative medicines

2020 has been a busy year for PNI, which announced a number of partnerships with pharma companies and contract manufacturing organizations (CMOs) to out-license its LNP and manufacturing technologies, while seeking partnerships with technology providers in the bio-processing field to provide complementary processing solutions.

In May 2020, PNI announced several partnerships to co-develop or manufacture nanomedicines, for example, a partnership with Entos Pharmaceuticals to give it access to the NanoAssemblr GMP system to manufacture a pan-coronavirus DNA vaccine. In addition, CanSino Biologics and PNI announced an agreement to co-develop an RNA vaccine against COVID-19. In June 2020, Sirnaomics and PNI signed a NanoAssemblr platform license and supply agreement to develop and manufacture RNA interference (RNAi) drug candidates.

PNI also partners with CMOs to provide NanoAssemblr platform users with access to the GMP system in a GMP environment. In March 2020, PNI announced a partnership with Fujifilm for the development and manufacturing of nanoparticle-based therapeutics. Fujifilm will use NanoAssemblr technology and instruments for in-house development and contract manufacturing services. AjiBio Pharma and Evonik are also authorized to provide contract services using PNI's manufacturing platform.

With its LNP delivery system, NanoAssemblr technology and its extensive drug development expertise, PNI's platform is ushering in the next wave of medicines in infectious diseases, cancer and rare diseases. PNI continues to seek high-value partnerships with leading drug developers, CMOs and technology partners.

1. Webb, C. et al. *Int. J. Pharm.* **582**, 119266 (2020).

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