

RESEARCH BEYOND BORDERS



Research Beyond Borders (RBB) acts as a 'radar' for the next big wave of innovation by fostering emerging therapeutic concepts and technologies. We are committed to advancing drug discovery for diseases with high unmet medical need. We offer excellence in drug discovery capabilities and expertise to forge collaborations with innovators and bring breakthrough therapies to patients in need.

Specific partnering priorities:

Gene Therapy: Tissue selective AAV-based therapy with breakthrough and translational potential for patients; innovative technology, which allows spatio-temporal control of cargo expression, increased cargo size or modulation of immune response to AAV capsids and/or DNA cassettes; human tissue/organ models for translational screening or characterization of AAVs; novel therapeutic concepts/targets, which are amenable to AAV-based gene therapy.

Regenerative Medicine: Focus on biology underlying endogenous mechanisms & master switches to regenerate tissues. The proposed therapeutic concept should be supported by preclinical *in vivo* data with a clear translational path to patients. Therapeutic fields of interest include, but are not limited to, tissues such as liver, pancreas, muscle, inner ear hair cells, bone, cartilage, skin and the thymus.

Emerging Therapeutic Concepts: Open to new and disruptive ideas in uncharted therapeutic spaces to treat diseases for which there are no effective therapies, including rare diseases.

New Therapeutic Technologies: Capture emerging technologies/modalities that will change medical practice e.g. extracellular vesicles.

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