

# Genzyme Pharmaceuticals

## RESOURCEFUL TOOLS FOR DELIVERING OLIGONUCLEOTIDE-BASED THERAPEUTICS

Oligonucleotide-based therapeutics are one of the most promising approaches in disease treatment today, however the delivery of RNA and DNA derivatives into the cells of the target organ presents substantial challenges. Genzyme Pharmaceuticals provides value-added solutions for delivering such actives using a unique combination of products, services, technologies, and strategic relationships.

### PRODUCTS

Genzyme Pharmaceuticals offers a variety of readily available products that are used in liposomal and other lipid-based delivery systems:

- > Synthetic phospholipids
- > Cationic lipids
- > Sphingolipids
- > "Helper" lipids

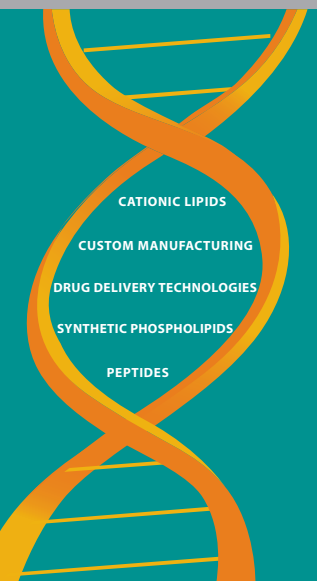
### CUSTOM MANUFACTURING SERVICES

- > Genzyme Pharmaceuticals' core competencies in custom manufacturing include:
  - > Lipids
  - > Peptides
  - > Other small molecules
  - > Polymers
  - > Carbohydrates
  - > Lipo-peptides and other conjugates
- > R&D: Selection and optimization of synthesis routes and development of scalable processes
- > Custom Manufacturing: Efficient transition into cGMP production from pre-clinical to commercial scale
- > CMC Support: Step-wise implementation of analytical and regulatory support required for filing

### DRUG DELIVERY TECHNOLOGIES

Genzyme Pharmaceuticals offers two proprietary lipid-based drug delivery technologies that may be considered for oligonucleotide delivery:

- > LipoBridge<sup>®</sup> is a technology based on small lipid-like molecules that facilitates transport of active drugs across the blood-brain barrier and into the CNS via the tight junctions.
- > LipoMask<sup>™</sup> is a technology based on small lipid-like molecules that, when used in conjunction with other particle-forming lipids, forms liposomes or nanoparticles that increase the circulation half life of actives in blood serum. LipoMask can be developed for both passive and active targeting.



## STRATEGIC RELATIONSHIPS

Genzyme Pharmaceuticals offers a series of strategic relationships designed to provide synergies with our core competencies:

### > Pharmidex, Ltd.

Cerense<sup>SM</sup> is a unique product development platform that combines Genzyme Pharmaceuticals' LipoBridge<sup>®</sup> nanotechnology with Pharmidex's capability to assess both in vitro and in vivo blood-brain barrier permeation through ingress, distribution and pharmacokinetics of lead compounds in the brain.

### > Brookwood Pharmaceuticals

Design for Peptide Delivery<sup>SM</sup> is a unique peptide delivery concept developed in conjunction with Brookwood Pharmaceuticals. By combining Brookwood's specialized knowledge of formulating peptides for long acting systemic delivery with Genzyme Pharmaceuticals' knowledge of peptide modification, clients obtain optimized peptides at an early stage for subsequent sustained delivery in the clinic.

### > Mimotopes

From Mimotopes' specialty in peptide libraries and candidate selection to Genzyme Pharmaceuticals' cGMP manufacture of the peptide Active Pharmaceutical Ingredient, the Mimotopes - Genzyme Pharmaceuticals Global Alliance provides seamless technology transition for customers at every step in the peptide drug development continuum.



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