

Acuitas Therapeutics

NON-CONFIDENTIAL PRESENTATION

November 2013



Company Background

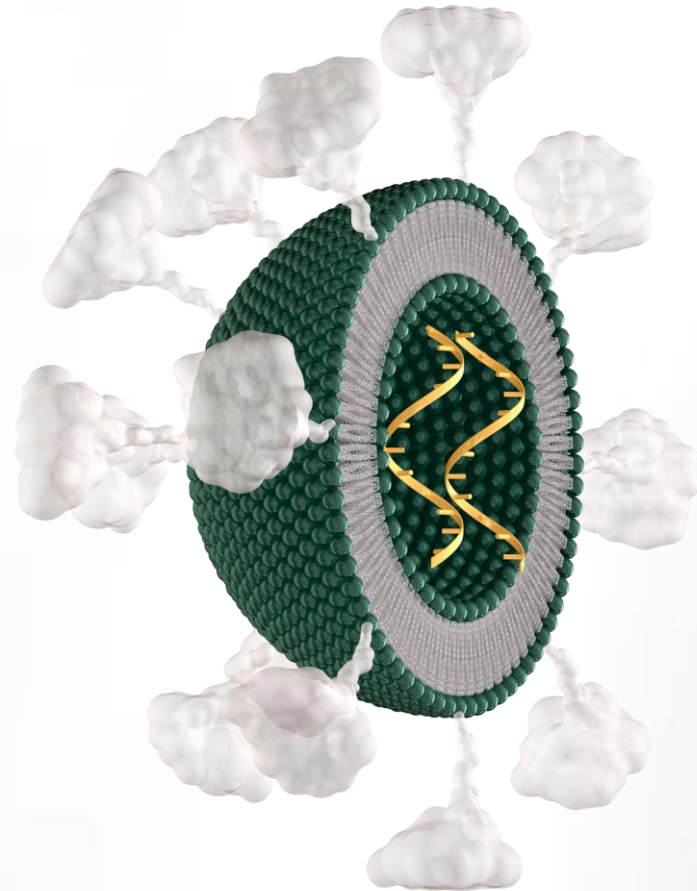
- ▶ Founded February 2009, based in Vancouver, British Columbia
- ▶ Formerly AlCana Technologies Inc
- ▶ Highly experienced team developing lipid nanoparticle delivery systems
- ▶ Facilities for chemistry, formulation and preclinical studies with access to additional resources at the University of British Columbia (UBC)

Technology Focus

- ▶ Systemic lipid nanoparticles for intracellular delivery of molecular therapeutics – primarily nucleic acids
- ▶ Pharmaceutical applications:
 - ▶ Protein replacement therapeutics (mRNA or plasmid delivery)
 - ▶ Protein knockdown therapeutics (antisense)

mRNA Lipid Nanoparticles

- ▶ Multi-component carrier
- ▶ Small, uniform sized particles (~80 nm)
- ▶ Low surface charge in blood compartment



Protein Replacement Therapeutics: mRNA Delivery

Proof of Concept Studies using reporter mRNA

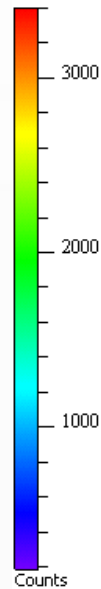
- ▶ Luciferase mRNA encapsulated in LNP – encapsulation efficiency >90%.
- ▶ In vivo administration (i.v.) to mice followed by:
 - ▶ Quantitative luciferase expression determined ex vivo (Steady-Glo Luciferase assay kit: Promega)
 - ▶ Live imaging (IVIS system)

mRNA Delivery

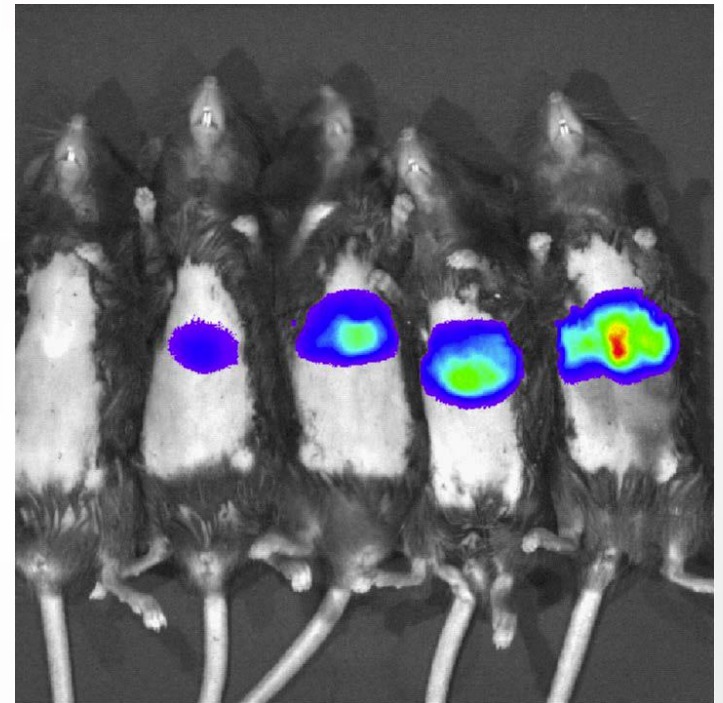
DOSE RESPONSIVE PROTEIN EXPRESSION

- ▶ Efficient mRNA delivery to liver.
- ▶ Luciferase expression proportional to mRNA dose (0.1-3.0 mg/kg)

Image
Min = -16
Max = 3635



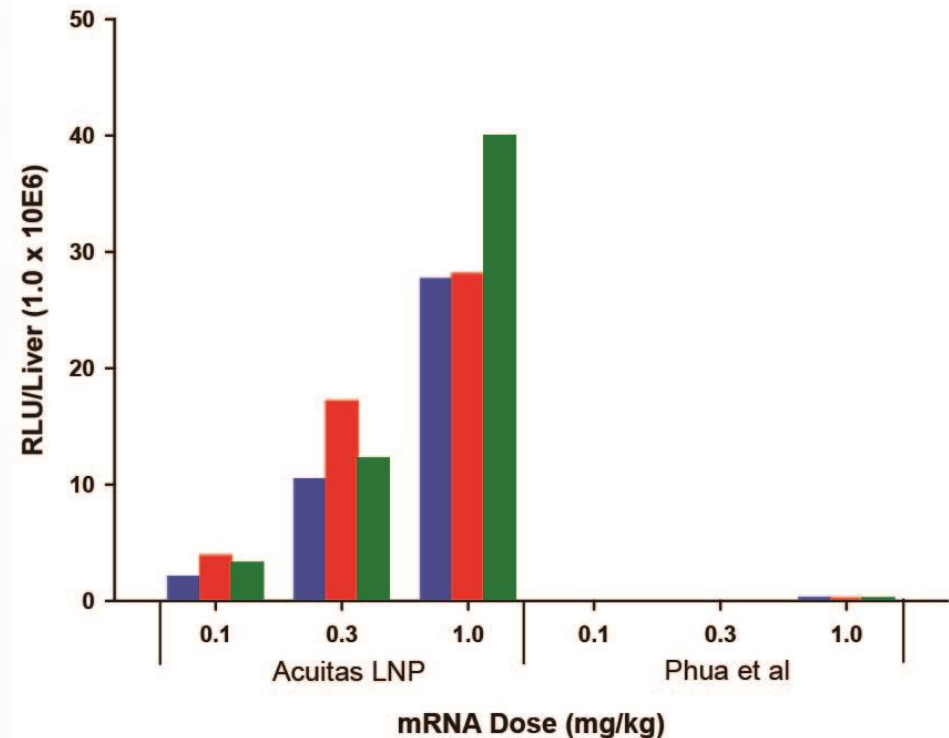
Color Bar
Min = 182
Max = 3400



mRNA Delivery

COMPARATIVE LNP POTENCY

- ▶ Acuitas LNP vs Phua et al. LNP¹
- ▶ Luciferase expression in liver 4 hours after iv administration



¹Phua, Leong and Nair, J. Control Release, 166(3) 227-233, 2013

Partnership Goals

- ▶ Focus on partnerships in protein replacement therapeutics
- ▶ Acuitas supports:
 - ▶ Preclinical screening to select Lead Products
 - ▶ Protection of Lead Products through new and existing IP rights
 - ▶ Analytical, biophysical and preclinical characterization of Lead Products
 - ▶ Transition from preclinical to clinical development

Contact Information

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