Background

• Geoff Crouse, MBA/MPH
  – 10 years senior management experience in Life Science and Diagnostic
  – Most recently COO of Immucor, blood typing diagnostic company in Atlanta

• Ian Copland, PhD
  – Assistant Professor Hem/Onc
  – Manager of Emory Personalized Immunotherapy Center
  – Co-Inventor of Product
Executive Summary

- A novel technology for the processing of human platelets
- Strong supply of low-cost raw material
- Platelet lysates are rich in growth-factors and anti-inflammatory factors
- Potential utilization in topical and injectable applications
- Market research complete and initial indication of use determined
Current PRP Market

- No allogeneic source of platelet rich plasma
- Autologous PRP market nascent with early data suggesting efficacy in a broad range of indications
- Primary utilization and clinical research in sports medicine applications
- Various companies selling fractionation equipment and PRP kits with 510(k) approval
- Some data suggests patient self-administration and physician utilization in KCS
- 40 patient study in ocular ulcers showed autologous PRP promoted healing and reduced inflammation
- Method of action and benefits of growth factors and anti-inflammatory factors within PRP are not well studied
Potential Indications of Use

Wound Care

• Large and growing market in chronic wound ulcers.  
  3-6M patients with $5-10B annual cost

• Difficult to improve outcomes due to concomitant peripheral perfusion deficiencies and lack of complete wound healing

• Topically applied platelet derived growth factors have been widely studied (PDGF)
  
  • Regranex is derived from recombinant factors
  
  • Centrifuge blood to isolate platelets and add thrombin (Curative Technologies - remote autologous processing, Autologel and SafeBlood - bedside processing)

• Potential to work with DOD on severe wound care

Sports Medicine

• Current market for autologous PRP is estimated at $40M annually - most common use

• Considered by many as "snake oil" due to lack of clinical data to support utilization

• Physicians income stream from self-pay market
KCS Indication
Keratoconjunctivitis Sicca ("KCS") or Dry Eye

• Market for ocular drugs and eye care products $12B

• Dry-eye prevalence 4.9M in US population over 50 years old

• Market estimated for Dry Eye drugs estimated at 10% growth and ~$1B in 2015

• Therapeutics in Dry Eye space have been difficult to bring to market due to multi-factorial disease, various levels of severity and difficult endpoints

• Most successful drug in the space is Restasis with $620M sales in 2010
  
  • 15% efficacy measured by increased tear productivity (vs 5% Endura)
  
  • 17% of patients experiencing severe ocular burning side-effect in 1200 patient study

• We will segment the patient population to focus on severe KCS (co-morbidities) to increase likelihood of statistically significant clinical improvement within a limited patient population
Preparation of Human Platelet Lysate

Plateletpheresis

1x10⁹ Plt/mL

5-7 days at 22°C

-80°C

1st Freeze
Thaw Fracture

Pooling of 5-10 plateletphereses. Aliquot and freeze -80°C

2nd Freeze/ Thaw Fracture

Centrifuge 8000xg

Progressive filtration 70 - 0.2 μm

Platelet Lysate

Platelet bodies (fragments)

Lot Testing
Sterility
Mycoplasma
Elisa (PDGF, TGF-
®)

Fibrinogen Depletion.
Human Platelet Lysate Processing

- Proprietary process developed with patent pending
- Completely acellular product with no debris
- Fibrinogen depletion prevents clotting allowing growth and anti-inflammatory factors to be
- Consistency across lots vs lack of control and variability in autologous processing
- Raw material comes from healthy repeat donors that are confirmed negative for infectious disease markers
Engagement with Emory Eye Experts

• Dr. Tim Olsen: Chair Emory Eye Center
• Dr. Bhairavi Dholakia: Clinician Scientist
• Dr. Henry Edelhauser: Pre-clinical Specialist
  – Mentored developer of Restasis
• Dr. Benard McCarey: Pre-clinical Specialist
  – Regularly tests solutions and devices of Ophthalmology product manufacturers
• Already developed strong relationships with FDA reviewers in Ophthalmology (i.e. Wiley Chambers Head of Pharmacology)
Recommendations by Eye Experts

• KCS is the right indication. Multiple secondary indications also possible. (i.e. Lye Injury, corneal repair follow surgery etc.)
• No good animal or in vitro models for Dry Eye or KCS
• May not require animal/in vitro efficacy to initiate Phase I trial
• In vitro models are not good predictors of toxicology
• FDA will require we perform a modified Draize test
• Better understanding of stability profile, pH, osmolality, dosage and diluent need to be defined
Next Steps

• Apply for GRA Phase 1B grant to fund pre-clinical work
  – GRA Phase 1A grant used to complete technical work and market research
• Recruit key members from Emory Eye Center in advisory capacity
• Complete pre-IND work for KCS indication by April 2012
• Supplement Patent Application prior to May 2012
• Complete business plan and determine commercial funding pathway in summer of 2012