



Combined Business Plan

Gene Regulation Therapy-Sickle Cell, Inc. (GRT-SC) and The Sickle Cell Cure Foundation, Inc. (SCCF)

Executive Summary

IN A NUTSHELL

Last year an estimated 340,300 infants were born with sickle cell disease (SCD) – mankind’s most commonly inherited health disorder. Ninety-five per cent of these neonates reside in the tropical malaria “belt” and the eastern Mediterranean. Most will die by age five. The current “gold” standard of treatment can indeed extend the lives of SCD victims to 40-45, but their families must be able to pay for lifelong sophisticated care only available in advanced economies and costing half a million dollars per victim.

Peer-reviewed laboratory trials documented here and abroad demonstrate that the patented cure is 100% effective and presents no side effects. Victims, families, and their governments will gladly pay for the cure as it is easier to administer, provides the desired medical outcome, doubles life expectancy to 80-85 years, costs 4.0% of the current standard treatment, bolsters national productivity, reduces welfare rolls, and addresses the root cause of the disease (genetics). For over a century, a genuine cure for SCD eluded the world, until now.

Already 11 countries have awarded patents to the co-founders of SCCF for their genome-based SCD cure. \$5.3 million in seed capital and 57 months of a seasoned manager can help to bring this long-awaited break-through to Phase III field trials and commercialization and to relieve humanity of this age-old scourge once and for all.

COMPANIES OVERVIEW AND BACKGROUND

Organized July 2006, SCCF is a 501(c)(3) (tax-exempt) non-profit, bio-medical research foundation in its administrative start-up phase. The SCCF board intends to partner with a more seasoned charity and expects to contract for Phase III field services to attract the necessary capital and management talent to commercialize the SCD cure in exchange for royalty flows from a pharmaceutical.

PRODUCT AND TECHNOLOGY

The key component of the cure is ferritin-heavy chain (FtH), a protein that occurs naturally in the body. FtH deactivates the mutant sickle cell gene and reactivates a dormant, healthy replacement gene. The cure is specific to the ailment and easy to administer. The drug’s molecular composition, clinical applications, and salutary effects

are proprietary and patent-protected. Quite stable, it requires no refrigeration – a definite “plus” in remote, tropical distribution networks. We intend to use our patented technology known as **Gene Regulation Therapy** (GRT) to control gene expression without permanently altering any genes – a medical first.

GRT-SC and its pharmaceutical awardee will, in theory, be able to eliminate the expression of SCD worldwide, if it were not for two countervailing factors: (1) logistical or administrative barriers in reaching remote populations and (2) the absence of government-mandated newborn screening in third-world countries.

PREDICTED BENEFITS FOR SCD VICTIMS AND GRT-SC INVESTORS

- Double the SCD patient life expectancy from 42 to 85 years (U.S. actuarial tables).
- Save an estimated 340,300 SCD-positive infants per year and after 16 years, some 8.4 million will have been saved.
- Reduce SCD medical bills by 96%. \$578 FtH treatment per patient year = 4.0% of standard full care of \$14,443.
- Reach 144.5% internal rate-of-return (IRR); generate \$396 million net present value (NPV) applying an 18% discount rate.
- 1X Break-even in 1 year 4 months; 2X in 2 years 1 month ; 5X in 4 years 9 months.

IRR and NPV calculations based on start-up costs of \$5.259 million (2009-2012) plus \$12.0 million in 2013 from pharmaceutical company, with 15 consecutive years of sales (2013-2028) grossing \$223 million during 2028 the final year. The biggest “unknown” cost factor is the time-consuming U.S. Patent Office and Food & Drug Administration (FDA) regulatory process. Our business plan assumes FDA will grant approval by December 2012 with sales beginning 12 months thereafter.

MANAGEMENT – “THE TEAM”

- Chairman & Founder Robert Broyles, PhD – President of SCCF, Professor of Biochemistry & Molecular Biology, Principal Investigator and specialist, discoverer of the SCD cure. Strengths: comprehensive scientific analysis and biomedical grant management. BS chemistry and PhD biochemistry - Wake Forest, National Institute of Health post-doctoral fellowship. Community service: American Red Cross, Boy Scouts, First Unitarian Church - Oklahoma.
- Director of Development (designate) Gary Bricker^{CFP} – Third World health finance specialist. Strengths: financial feasibility and program management. political science studies – la Sorbonne, BA - economics University of Connecticut, MS - urban planning Columbia University. USAID (Zambia, Somalia, Indonesia, El Salvador, Tunisia, Georgia, Morocco) – 24 years. Community service: School without Boundaries, Black Sea University, First Unitarian Church - Oklahoma City.
- CFO & Director of Development, To Be Determined. CPA and/or MBA. At least five years of progressively responsible experience in fund raising (grants, loans, venture capital, and “S” corp shares). Project incubator skills, advice on corporate tax, legal requirements, and filings. Strengths: objective personnel assessments, leadership

skills, and team building advice. Commitment to the humanitarian goals of the SCCF and GRT-SC. A vision of commercialization that goes *beyond "for-profit" only*.

MARKET OPPORTUNITY

Market size of SCD patients has remained historically small (107,000 patients, US & Europe) due to low survival rates. With the introduction of the cure, the contingent of sickle cell survivors could grow by an estimated 340,300 each year. A potential competitor, Bristol-Myers Squibb (BMS), the manufacturer of hydroxyurea (HU), has not pursued a genetic path to an SCD cure. We predict BMS will not resist our market entrée, since the proposed cure is superior to standard care both medically and financially. XEchem, Inc. of Nigeria has relevant FDA patent applications pending. Our understanding is that its product Nicosan™ is palliative in nature and does not function through gene regulation.

In exchange for royalty payments, we intend to negotiate an exclusive licensing agreement with a competitively selected pharmaceutical company (or consortium) to design and fund Phase III trials and begin marketing as soon as 12 months thereafter. Reported innovations to accelerate FDA review procedures could shorten the 57-month start-up period, thereby strengthening financial ratios, and increasing investors' return-on-investment. We would prefer licensing to an American-registered company but are actively considering European options in view of our existing European patents and Europe's established drug distribution networks into Africa where 75% of the SCD caseload resides. Year 1 marketing would focus on Europe and the Americas, the two highest income WHO regions, to allow for a cross-subsidy approach to take hold.

FUNDS SOUGHT, FINANCIAL PROJECTIONS, AND EXIT STRATEGY

We seek aggregate commitments of \$5.3 million in grants, venture seed capital, discount rate loans, and/or commercial promissory notes disbursed against pre-agreed performance-based draws for up to four years.

In a gesture of corporate social responsibility, our licensing agreement will commit the winning pharmaceutical company to lower or maintain prices steady as long as possible. We will also encourage GRT-SC shareholders to contribute generously to the SCCF research agenda. Liquidation may occur only after markets demonstrate pre-agreed measures of predictability and stability and after consideration of post-patent generic options.

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