Executive Summary

ZATA, Inc. - Novel Oligotherapeutics

September 2013

THE COMPANY

ZATA Pharmaceuticals is a start-up business that was founded in 2008 and has been structured as a privately held C-Corporation. The Company's technology vision is the development of platform technology that enables a novel class of oligotherapeutics (**OTs**), the synthetic compounds for the treatment cancer, infections, and genetic diseases as well as scarless wound healing. Preliminary data indicating the feasibility of ZATA technology has been obtained. The concept behind the technology, IP, technical capability, and medical need of ZATA's OTs has been highly praised by independent experts at NIH and MassBio. ZATA has organic synthesis facilities and BL2 level laboratory space in a specially designed incubator style biotechnology research unit. ZATA owns all the necessary basic research devices and has the capacity to synthesize and test nucleic acid derivatives at the tissue culture level.

PRODUCTS

Management's ultimate goal is to provide the finest in product quality relative to price, providing the customer with true value. The product offers a large number of OTs (RNA/DNA therapy products based on synthetic oligonucleotide derivatives) that can be applied across a wide range of therapeutic areas, as mentioned above. ZATA's Business model targets early and long-term products development enabled by company's technology.

ZATA's **early products** will comprise OTs targeting three diseases including HIV, hepatitis C, and malaria, which will be developed up to phase I clinical studies. Management plans to license these OTs and related IP to pharmaceutical companies for further development and commercialization. License deal will include upfront plus milestone payments which will provide ZATA strong and stable long-term financial portfolio. Such business approach will provide two major advantages a) will enable the strong base for the ZATA's platform technology that makes it easy to develop other OTs and b) gives ZATA flexible financial leverage. Management envisions completion of the early product development cycle within the first three years.

Licensing revenue will allow ZATA to expand its internal pipeline, to development and commercialize **long-term products,** such as OTs against prostate cancers (PC) and for scarless wound healing. OTs for treatment of PC and scar prevention could be administrated focally which provides the great advantage for high therapeutic efficacy. Parallel to that, the company will continue developing and licensing additional OTs for a number of other diseases (there are over two hundred different cancers for instance).

MARKET

The market opportunity is substantial, since this class of novel OTs can in principle be applied across a wide range of therapeutic areas. ZATA envisions that beneficiaries for the proposed drugs will be patients with cancers and patients with infectious and genetic diseases. Examples with high unmet medical needs are two hundred different types of cancers, HIV, hepatitis C, malaria, diabetes (type I), TB, Cystic Fibrosis, Huntington and *Alzheimer's disease*, prevention of post-surgical scaring and others.

The broad target market for the first three years for ZATA product and IP includes big and small therapeutic companies involved in development and commercialization of RNA/DNA therapy products as well as academic research institutions. An initial list of websites of the target companies can be seen at: http://biopharmguy.com/links/company-by-location-dnarna.php ZATA will consider collaboration with one of the pharmaceutical companies with established worldwide market infrastructure for the set-up of manufacturing and selling capabilities of OTs that can be developed by ZATA internally.

The pharmaceutical and medical manufacturing industry has experienced a recent growth rate of 1.58% despite the continued national economic downturn as the population ages. The medical industry seems to be one of the few industries not effected from the declining economy. According to the National Center for Health Statistics (cdc.gov), there are 97,700,000 Americans suffering from chronic illnesses that can be candidates for ZATA Pharmaceuticals products, in addition to every American who contracts the influenza virus each year. They include: 23.6 million cancer sufferers; 3.0 million arthritis sufferers; 15.0 million diabetes sufferers; 25.0 million chronic liver disease sufferers and 1.1 million HIV sufferers.

COMPETITION AND BUYING PATTERNS

In general, competition within the industry is fierce and intense. The industry competitors are facing many of challenges which creates a window of opportunity for ZATA Pharmaceuticals. There are several large pharmaceutical companies interested in the development of oligotherapeutics (**Novartis, Pfizer, Merck, Sanofi, Eli Lilly**, and others). There are also a few dozen other companies which are focused ONLY on the development of oliogotherapeutics, such as **Alnylam Pharmaceuticals** (products: siRNA, RNAi), **ISIS Pharmaceuticals** (products: Antisense), **Regulus Therapeutics** (products: microRNA), **Dicerna** (products:

RNAi), **Marina Biotech** (products: RNAi), **Antisense Pharma** (products: Antisense), **RXi** (products:siRNA, RNAi), **Noxxon** (products: Spiegelmers, non-natural L-RNA), **Sarepta Therapeutics** (products: Phosphorodiamidate Morpholinos), **miRagen Therapeutics** (products: microRNA), **Santaris Pharma** (products: microRNA), **Moderna** (products: mRNA), **Sirna Therapeutics** (products: siRNA) and others. Description of the products these and other companies in the field are working on can be found on their web page list, which is provided on the link: http://biopharmguy.com/links/company-by-location-dnarna.php.

Some of them have been in business for over two decades. However, there are only three drugs on the market; **Vitravene**® (ISIS) – CMV for HIV+ patients, **Macugen**® (OSI EyeTech) – Wet AMD **Kynamro®** (ISIS, Sanofi) – hypercholesterolemia. One reason for such a low number of commercialized products is that none of the currently used OTs fully satisfies the criteria of optimal OTs. One such criterion is cellular uptake. Cell membranes do not allow for the cellular uptake of negatively charged OTs currently used. ZATA's technology enables the production of neutral OTs which enhances the cellular uptake and consequently therapeutic efficacy as outlined in the technology section below.

TECHNOLOGY

This technology will enable a robust nucleic acid chemistry platform allowing for the production of novel OT capable of penetrating the outer cellular membrane without formulation. The technology includes: 1) A novel class of **OTs** synthesized via phosphoramidite chemistry that permit facile attachment throughout certain branched chemical groups that bear positive charges at their termini; 2) Length optimization of each branch to allow positive charges to reach neighboring phosphate groups and neutralize their negative charges; 3) Introduction of partial hydrophobic properties in the backbones of the ON at the sites where the aforementioned branched groups will be incorporated; 4) Proven retention of thermodynamically stable and chemically specific hybridization to targets by these novel ON. **These unique features have been demonstrated to apply equally to all currently known oligotherapy approaches.** Figure 1 schematically illustrates a hybridized ZATA OTs to target gene (to mRNA).

OTs enabled by ZATA's platform technology will be suitable for all currently used oligo therapy approaches, such as siRNA, miR, and antisense. Technology will also enable targeting and down regulating several harmful genes simultaneously. For instance, for the prevention of prostate cancer proliferation several known oncogenes, such as TP53INP1, survivin, Bcl-2, and CK2 mRNAs can be targeted and down regulated with ZATA's OTs. Cancer prevention could also be achieved by targeting large number of micro-RNAs (miR) that facilitate tumor cells growth.

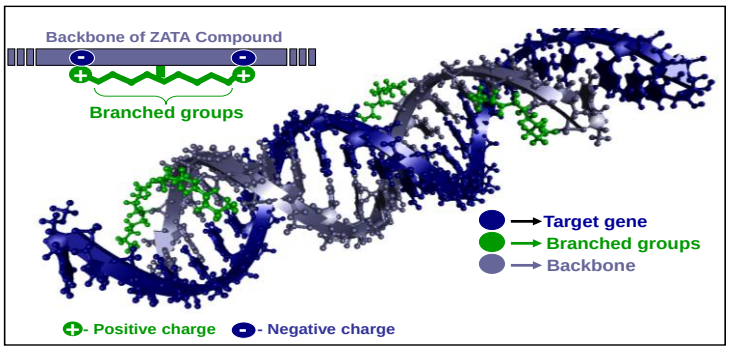


Figure 1. *Novel features of ZATA Compounds. Shown is a target RNA (Blue) to which a ZATA OT (grey) with branched groups (green) in their backbones has hybridized. Note the preservation of the typical double helical structure of a DNA:RNA hybrid or RNA:RNA duplex*

COMPETITIVE ADVANTAGES

ZATA's competitive advantage is based on strong IP covering a broad platform of methods of use and composition of matter. Molecular structures of ZATA compounds will enable self-delivery without special formulation. Only ZATA's structures satisfy all necessary criteria toward optimal OTs. These complex criteria can be summarized as a set of three general properties: 1) a low number of charges, introduction of some degree of hydrophobicity across the backbones, and water solubility, all of which would **enhance cellular uptake**: 2) OTs must maintain natural hybridization properties, have efficacy at the target gene, be stable toward nucleases, and be relatively non-toxic, which will provide for **therapeutic efficacy**, and improve the therapeutic index: 3) time, labor, and cost-effective method of synthesis, that will allow **scale-up** and economic viability.

This technology is protected by a new patent application. ZATA is also in negotiation with UMass Medical School (Worcester, MA) to license issued patent (Patent – 8084589) invented by scientists affiliated with ZATA. The proposed technology is based on the scientific legacy of the founder of the field, Dr. Paul Zamecnik, one of the cofounders of ZATA (link about Dr. Zamecnik's legacy: http://www.ncbi.nlm.nih.gov/pubmed/15952879). ZATA's approach can succeed where others failed.

IP

Patent, provisional patent application, and literature describing preliminary data are listed in addendum 1.

INDIPENDENT EXPERT OPINIONS

In April of 2013 NIH Scientific Review Group (independent government body that evaluates the merit of emerging biotech projects) concluded that ZATA's technology is:

- **Science -** "straightforward and promising"
- **Approach -** "clever concept"
- **Team -** "fully capable of successfully executing the proposed technology"
- **Equipment–** "ZATA is well equipped to carry out proposed work"
- **Significance** - "Improved delivery of OTs into cells will greatly increase the therapeutic potential of OTs

On Mass-Challenge 2013 Accelerator contest, ZATA's project describing potential anti-scaring OTs made it to the semi-final round.

In October 2012 ZATA was selected among three companies as "Fast-Forward Potentially Groundbreaking Work" by experts at Mass Biotechnology Councils**.**

Based on 2012 NIH reviews, ZATA's project is rated within the top 10% of biotech projects.

TECHNOLOGY DEVELOPMENT STRATEGY

ZATA's first major milestone is the validation of platform technology in several disease models that may enable the licensing of ZATA's IP for the development of specific OTs for the treatment of those diseases. Such a business model may enable ZATA to obtain solid revenue for its internal pipeline. ZATA's management envisions achieving that milestone within three years of technology development. Diagram 1 below demonstrates three stages of ZATA's technology development.

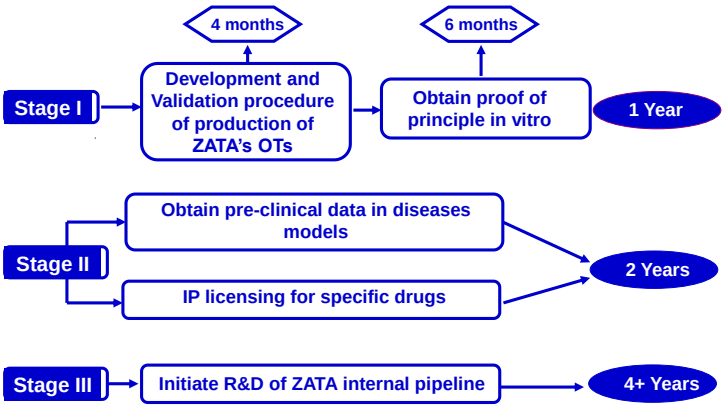


Diagram 1. *Three stages of ZATA's technology development*

Management's goal during these few years is to solve the problem with cellular uptake of oligotherapeutics and establish a viable OTs company.

Stage I comprises the development and validation methods of synthesis and formulation of ZATA's OTs as well as obtaining solid in vitro data indicating enhanced cellular uptake vs. currently used OTs. Stage I can be completed within 1 year.

In Stage II, management of ZATA aims to obtain preclinical data in different disease models, establish a custom service capability, and expand the IP portfolio which can initiate IP licensing for specific drugs (OTs) for the treatment of specific diseases. Stage II can be completed in two years.

Stage III comprises initiation of R&D of ZATA's internal pipeline which will depend on the revenue obtained at the end of the Stage II and therefore is not addressed in detail in this summary.

Diagram 2 below is a road map for Stage I of development consisting of four sequential steps after obtaining working capital: Step 1 - we will obtain commercially available and custom synthesized (tentatively from GLSynthesis, Inc.) chemicals (2 months): Step 2 - in the following three months we will prepare several of ZATA's compounds and controls: Step 3 - in another three months we will demonstrate the effective cellular uptake of non-formulated ZATA compounds: Step 4 - in the last two months we will demonstrate effective inhibition of target mRNAs in a cell free system. Such data will be sufficient for biological and chemical validation of ZATA's OTs.

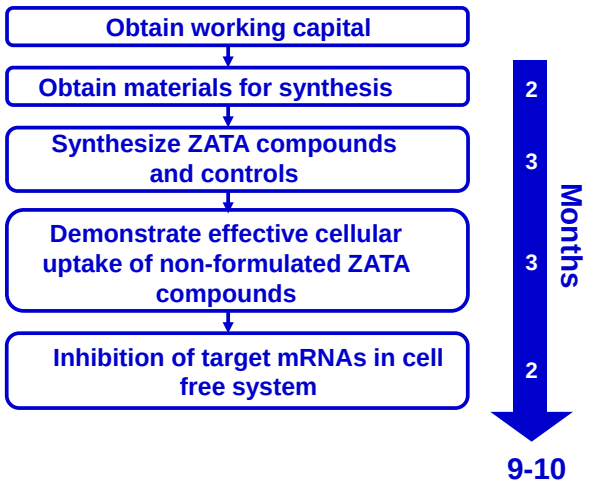


Diagram 2. *Road map for stage I of technology development*

Diagram 3 below is a road map for Stage II of development which consists of three sequential steps: management has selected four disease areas that we have previous experience with to focus on first; cystic fibrosis, HIV, malaria,

and prostate cancer. Step 1 - Synthesize in-house ZATA compounds targeting key target genes implicated in the pathogenesis of these diseases (6 months); Step 2 - perform animal studies for ZATA compounds over a period of 14 months in relevant disease models and Step 3 - transfer developed compounds and license IP to pharmaceutical companies including upfront payments. In addition to licensing the IP in specific areas, ZATA will also offer to corporate partners the development of ZATA OTs for the disease models they are interested in. Development of Stage II will be complete in a period of two years.

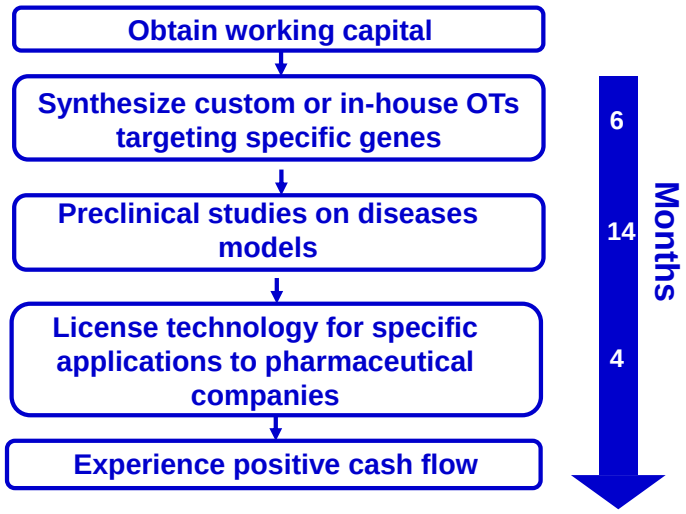


Diagram 3. *Road map for stage II of technology development*

Time frame and need of working capital for Stage III of development will depend on licensing revenue after completion of Stage II.

FINANCIAL CONSIDERATIONS AND COMPARABLES

The financial forecast within this summary includes obtaining a capital injection through a financing source for the first two stages of technology development, which will allow ZATA to obtain preclinical data in various disease models, and the licensing of IP for the development of OTs that target specific diseases will fund the third stage. Such an approach toward business development may enable ZATA management to obtain revenue from IP licensing, part of which could possibly be used to compensate the first round investors and company owners for their efforts. The rest will be invested in the development of ZATA's internal pipeline which correlates to Stage III (final) of development. To have maneuvering flexibility in Stage III of development, ZATA management considers only minimally necessary working capital for Stage I and II development will be required as indicated below.

As it is defined in "technology development strategy," ZATA's first key milestone is obtaining validated preclinical data in several disease models that may enable the licensing of ZATA's IP for the development of final OTs for the treatments of those specific diseases. ZATA anticipates receiving a lump sum upfront for licensing IP and shares from the future net profit of commercialization of those drugs. Such a business model will provide a stable financial portfolio for the long-term.

Seed capital for the set-up and start of operations of ZATA was provided by the founders of the company.

A year-by-year uses of capital is shown in Table 1. Specific expense numbers are available in ZATA's budgets for years 1 to 3 (Available upon request). ZATA anticipates licensing 2 to 4 OTs for specific diseases that may raise up to $55M upfront plus $1.2B in milestone payments. Summary financial projections for years 1 to 3 are outlined in Chart 1 below. Comparable examples are provided below in bullets:

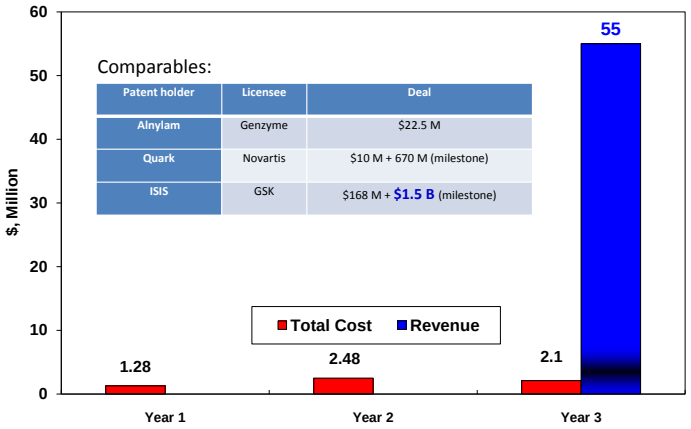
- Genzyme's payment to Alnylam $22.5M upfront fees to license their phase I siRNA drug (ALN-TTR02) in Asia.
- Quark got $10 M upfront and 670 M milestone payments from Novartis for an exclusive worldwide license to develop and commercialize siRNA drug QPI-1002, currently in a Phase II clinical trial.
- ISIS licenses antisense drugs to GSK for its orphan drug portfolio. Upfront payment $168M. The deal is valued at $ 1.5B inclusive of milestone payments
- Merck and Roche pays SiRNA $300+M upfront and 1.1B milestone for limited platform *license* from Alnylam

Table 1. *Requested funds for validation of ZATA's platform technology in disease models (Available upon request)*

Revenue from licensing IP may generate sufficient positive cash flow to compensate owners of company for their efforts.

For the development of ZATA's compounds we would consider the issuance of equity or non-dilutive funding. For equity funding we consider Angels, Venture Funds, and corporate Venture (Pharma). For non-dilutive we consider licenses to Pharma, Disease Foundation Grants, government grants and modest investments for non-exclusive options or observation rights.

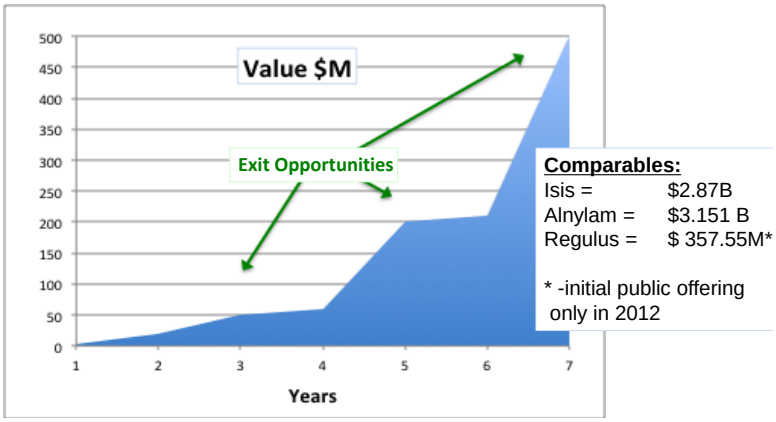
Chart 1. Highlights of ZATA's Financial Projections



Exit Strategy

The opportunity for a healthy return on investment is demonstrated on this Chart 2. There will be three major post-investment exit points around 3, 5 and 7 years respectively. The first exit point may provide a healthy return for short-term investments, while second and third exit points may provide even larger returns.

Chart 2. Opportunity for Healthy Return on Investment



As emphasized above and demonstrated in Table 1, ZATA's management plans to obtain minimally necessary working capital to reach its milestone and obtain IP licensing revenue for its internal pipeline. Management envisions obtaining limited second round of investments in exchange for company equity if revenue from IP licensing will not cover company's financial needs.

ZATA Pharmaceuticals, Inc. is seeking statements of interest from parties interested in licensing and/or sponsoring collaborative research to further develop, evaluate, or commercialize this technology.

MANAGEMENT SUMMARY

Company was co-founded by Dr. David Tabatadze and Dr. Paul Zamecnik in 2008. The management of ZATA Pharmaceuticals will have extensive knowledge of the industry and pertinent experience and training that will assist in the successful management of the company. Management will consist of a President/Chief Executive Officer, Plant Manager, Quality Manager, and a Production Manager. There will also be a board of directors with scientific and business experience and scientific advisors. The R&D team, scientific advisers, and board of directors have been identified.

CONTACT

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ADDENDUM 1

PATENT AND PATENT APPLICATION

1. Alexei Bogdanov, Valeriy Metelev, David Tabatadze and Paul Zamechnik (2011) Phosphoramidite nucleoside analogs; US Patent 8084589 - 8084589 (In process of licensing from UMass Med. School)
2. David Tabatadze (2013) PHOSPHORAMIDITE SYNTHON FOR THE SYNTHESIS OF NEGATIVE CHARGES REDUCED OLIGOTHERAPEUTICS Provisional patent application that protects the structures filed 03.29.2013

PUBLICATIONS COMPRISING PRELIMINARY DATA

1. Valeriy Metelev, Surong Zhang, David Tabatadze, and Alexei Bogdanov, Jr. (2013) The three-dimensional context of a double helix determines fluorescence of the internucleoside-tethered pair of fluorophores. *Molecular BioSystems.* Aug 27;9(10):2447-53. doi: 10.1039/c3mb70108e.PMID 23925269
2. Valeriy Metelev, Surong Zhang, David Tabatadze, and Alexei Bogdanov, Jr. (2011) Hairpin-like fluorescent probe for imaging NF-kB transcription factor activity. Bioconjug Chem. 20;22(4):759-65.
3. Tabatadze D, Zamecnik P, Yanachkov I, Wright G, Pierson K, Zhang S, Bogdanov A Jr, Metelev V. (2008) A novel thymidine phosphoramidite synthon for incorporation of internucleoside phosphate linkers during automated oligodeoxynucleotide synthesis. Nucleosides Nucleotides Nucleic Acids. 27(2):157-72.

4. Zhang S, Metelev V, Tabatadze D, Zamecnik P, Bogdanov A Jr. (2008) Near-infrared fluorescent oligodeoxyribonucleotide reporters for sensing NF-kappaB DNA interactions in vitro. Oligonucleotides. 18(3):235-43.
5. Zhang S, Metelev V, Tabatadze D, Zamecnik PC, Bogdanov A Jr (2008) Fluorescence resonance energy transfer in near-infrared fluorescent oligonucleotide probes for detecting protein-DNA interactions. PNAS, 105(11):4156-61.