

Trucode Gene Repair, Inc.

COMMON STOCK VALUATION

AS OF JANUARY 31, 2019

*Report Date: April 23, 2019*



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April 23, 2019

Ms. Lauren Frenz

Senior Vice President, Corporate Strategy & Finance

Trucode Gene Repair, Inc.

329 Oyster Point Boulevard, 3rd Floor

South San Francisco, CA 94080

Dear Ms. Frenz:

In response to the engagement letter, RNA Advisors, LLC dba RNA Capital Advisors (“RNA” or “we”) has completed an analysis of Trucode Gene Repair, Inc. (“Trucode” or the “Company”) as of January 31, 2019 (the “Valuation Date”), to determine both the fair market value and the fair value of the Company’s common stock (“Common Stock”) on a non-marketable, minority interest basis.

Please note that this letter along with the following report (the “Report”), exhibits (individually an “Exhibit” and collectively the “Exhibits”) and their conclusions (jointly, the “Valuation” or the “Opinion”) are intended for the use of the management and Board of Directors of the Company (“Management”). This analysis has been performed in recognition of Internal Revenue Code Section 409A (“IRC 409A”) and FASB Accounting Standards Codification Topic 718 – Stock Compensation (“ASC 718”) (formerly known as Statement of Financial Accounting Standards No. 123R, Accounting for Share-Based Payment (“FAS 123R”)). We make no representation as to the accuracy of this Valuation if it is used for any other purpose without the written consent of RNA. This Opinion should not be considered, in whole or in part, as investment advice by anyone. This valuation engagement was conducted in accordance with the Statement of Standards for Valuation Services No.1 of the American Institute of Certified Public Accountants (“AICPA”).

This analysis uses the methods and techniques outlined in the AICPA Audit and Accounting Practice Aid entitled Valuation of Privately-Held-Company Equity Securities Issued as Compensation, second edition (the “Practice Aid”), which are relevant to the valuation of the Common Stock.

The definition of fair market value is predicated on IRS Revenue Ruling 59-60.

This cover letter provides an overview of the purpose and scope of the analysis and its conclusions. Please refer to the attached Report below for a discussion and presentation of the analysis performed in connection with this engagement.

SUMMARY OF FINDINGS:

Based upon the information and financial data provided, and representations made by Management, as well as the analyses performed, it is our opinion that the fair market value and the fair value of the Common Stock on a non-marketable, minority interest basis as of the Valuation Date is reasonably stated as follows:

$0.60 (SIXTY CENTS) per Share

The conclusions and opinions expressed in this letter and the accompanying Report are contingent upon the qualifying factors set forth in the Statement of Limiting Conditions and throughout the completed Report.

If you have any questions concerning this Report, please contact me at 925.940.0220.

Sincerely,

**RNA Capital Advisors**



Sam Renwick, CFA

*Primary Valuation Analyst*

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Engagement Overview

Purpose

RNA has completed an analysis of the Company as of the Valuation Date to determine both the fair market value and the fair value of the Common Stock on a non-marketable, minority interest basis. This analysis has been performed in recognition of IRC 409A and ASC 718. This analysis uses the methods and techniques outlined in the Practice Aid, which are relevant to the valuation of the Common Stock. The definition of fair market value is predicated on IRS Revenue Ruling 59-60.

Scope

RNA has based this Opinion on information provided and represented by Management. Our review and analysis included, but was not necessarily limited to, the following steps:

1. Reviewed the Previous Valuation report (defined below);
2. Discussed the expected operations, financial condition, and future prospects with Management in order to understand the performance of the Company;
3. Reviewed the Company’s financial statements for the years ended December 31, 2017 and December 31, 2018;
4. Reviewed consolidated forecasts and projections prepared by Management for the Company;
5. Reviewed a capitalization summary of the Company as prepared by Management;
6. Reviewed a copy of the Company's Articles of Incorporation;
7. Reviewed copies of certain documents pertaining to various securities underlying the Company's capital structure, such as preferred and common stock;
8. Reviewed certain publicly available financial data for companies that we deemed comparable to the Company;
9. Conducted research concerning the economic conditions and outlook for the US economy generally as of the Valuation Date; and
10. Conducted other studies, analyses and inquiries, as we deemed appropriate.

RNA did not independently verify the information provided; therefore, the validity of our Opinion depends on the completeness and accuracy of the information provided to RNA by Management. Management warranted to RNA that the information supplied was complete and accurate to the best of its knowledge. Information furnished by the Company and others, upon which all or portions of our Opinion are based, is believed to be reliable and we have assumed that all facts and circumstances that would significantly affect the results of the Valuation have been disclosed to us. However, RNA provides no warranty as to the accuracy of such information. Our fee for this service is not contingent upon the Valuation expressed herein.

Key Definitions

The term “CAGR”, as used herein, refers to compound annual growth rate.

The term “Cas9”, as used herein, refers to CRISPR (defined below) associated protein 9.

The term “CRISPR”, as used herein, refers to clustered regularly interspaced short palindromic repeats.

The term “DCF”, as used herein, refers to discounted cash flow method.

The term “DLOM”, as used herein, refers to discount for lack of marketability.

The term “DNA”, as used herein, refers to deoxyribonucleic acid.

The terms “γ-PNA” or “gamma PNA”, as used herein, refers to the gamma-peptide nucleic acid.

The term “EP”, as used herein, refers to European Patent.

The term “FDA”, as used herein, refers to the US Food and Drug Administration.

The term “HIV”, as used herein, refers to human immunodeficiency virus.

The term “IND”, as used herein, refers to an investigational new drug (usually in the context of a filing).

The term “IP”, as used herein, refers to intellectual property.

The term “IPO”, as used herein, refers to an initial public offering.

The term “M&A”, as used herein, refers to mergers and acquisitions.

The term “PNA”, as used herein, refers to peptide nucleic acid.

The term “NP”, as used herein, refers to nucleoprotein.

The term “Previous Valuation”, as used herein, refers to the valuation of Common Stock performed by RNA as of October 31, 2018.

The term “R&D”, as used herein, refers to research and development.

The term “RNA”, as used herein, refers to ribonucleic acid or RNA Capital Advisors, as per the context.

The term “rNPV”, as used herein, refers to the risk-adjusted net present value.

The term “SCD”, as used herein, refers to the sickle cell disease.

The term “US”, as used herein, refers to the United States of America and its major territories.

The term “USD”, as used herein, refers to US Dollars. Unless otherwise noted, all currency figures in this Opinion are expressed in USD.

Standard of Value

Definition of Fair Market Value

For tax purposes, the appropriate standard of value is fair market value, which is defined as:

“The price, expressed in terms of cash equivalents, at which such property would change hands between a hypothetical willing and able buyer and a hypothetical willing and able seller, acting at arms’ length in an open and unrestricted market, when neither is under compulsion to buy or to sell, and when both have reasonable knowledge of relevant facts.”[[1]](#footnote-2)

Definition of Fair Value

For financial reporting purposes, the appropriate standard of value is fair value, which is defined as:

“The amount at which an asset (or liability) could be bought (or incurred) or sold (or settled) in a current transaction between willing parties, that is, other than in a forced or liquidation sale.”[[2]](#footnote-3)

According to the May 7, 2003, FASB Board meeting, the above definition of fair value may be consistent with the definition of fair market value in Internal Revenue Ruling 59-60. RNA is not aware of any facts that would cause a difference in the conclusions on a fair market value basis compared with the fair value. As such, it is not unreasonable that the conclusion of fair value for financial reporting purposes ought to be consistent with fair market value for tax reporting purposes.

Company Overview

Background

Trucode is a gene editing discovery-stage therapeutics company. The Company was originally known as CDF Therapeutics, Inc. (“CDF”) and post the merger with PNA Innovations, Inc. (“PNAi”) in February 2017, the Company rebranded itself to Trucode Gene Repair, Inc. in May/June 2017. The Company has a lead candidate targeting SCD, has ongoing mouse studies on *in vivo* gene editing and is planning to initiate the non-human primate study by September 2019.[[3]](#footnote-4)

Products and Technology[[4]](#footnote-5)

The Company’s triplex gene-editing platform delivers corrected DNA sequence and repairs disease-causing genes. The Company’s chemistry enables DNA strand invasion and activates native DNA repair. The drug product is an intravenous injection of nanoparticles containing γ-PNA and DNA. Sequence-specific γ-PNA induces the process of natural DNA repair. Preliminary safety results of the study on mice support γ-PNA as a safe molecular class.

The Company’s gene editing technology is analogous to CRISPR/Cas9 but is differentiated. The IP on triplex gene editing is exclusive as compared to IP on CRISPR/Cas9. Also, unlike CRISPR/Cas9, delivery in triplex gene editing is intravenous, there is no endonuclease to deliver or control, and the technology targets 1-10 base pairs. The Company’s triplex gene editing technology does not require bone marrow suppression or viral vector and does not require endonuclease to manufacture at scale, deliver or control post-delivery. The Company expects fewer off-target effects with triplex gene editing vs. CRISPR/Cas9. The Company also expects to further develop a gene-editing platform, using its technology.

As of the Valuation Date, the Company focuses on the development of its lead candidate for SCD. Based on its preclinical development trials, the Company identified five conformance batches that were able to impact SCD, exhibiting a gene-editing behavior in the SCD bone marrow cells. Management expects a lead PNA and NP selection by the end of June 2019 and expects to complete its *in vivo* animal studies on mice by the end of July 2019.

Developments Since Previous Valuation

1. The Company failed to yield positive results from its ongoing trials. As such, Management suspended the preclinical research efforts for cystic fibrosis and beta thalassemia indications and focused all its resources for the development of its SCD targeted program;
2. Management reduced its research expenses and employee headcount (Woburn facility was closed);
3. The Company reduced its monomer spend and subleased its additional space; and
4. The overall 2019 Company budget was reduced by approximately $10.1 million or 41.0%.

Intellectual Property[[5]](#footnote-6)

As of the Valuation Date, the Company had a broad IP portfolio. The Company’s owned patents and applications includes:

*Table 1: Company Owned IP*

|  |  |  |
| --- | --- | --- |
| TITLE | NO. OF PATENTS GRANTED | NO. OF PATENTS PENDING |
| Polymer-Based Nanoparticles Related Formulation Methods, and Apparatus | - | 2 |
| PNA Monomers with an Orthogonally Protected Ester Moiety | - | 2 |
| PNA Monomers with an Orthogonally Protected Ester Moiety and Novel Intermediates and Methods Related Thereto | - | 2 |
| PNA Oligomers and Related Methods of Synthesis and Purification Thereof | - | 1 |
| Lipid Nanoparticle Formulations  of Peptide Nucleic Acids | - | 1 |

Below is a brief description of the licensed IPs from the following universities:

*Table 2: IPs from* Carnegie Mellon University

|  |  |  |
| --- | --- | --- |
| TITLE | NO. OF PATENTS GRANTED | NO. OF PATENTS PENDING |
| Conformationally-Preorganized, MiniPEG-Containing γPNA | US – 3 | 4 |
| γPNA MiniPobes for Fluorescent Labeling | US – 1; EP – 1 | - |

*Table 3: IPs from* Yale University[[6]](#footnote-7)

|  |  |  |
| --- | --- | --- |
| TITLE | NO. OF PATENTS GRANTED | NO. OF PATENTS PENDING |
| Targeted and High Density Drug Loaded Polymeric Materials | US – 3,  International – 8 | 1 |
| Compositions and Methods for Enhancing Triplex and Nuclease-Based Gene Editing | - | 1 |
| Compositions and Methods of Using Plerixafor to Enhance Gene Editing | - | 1 |
| Pseudo-complementary Oligonucleotides for Targeted Gene Therapy | US – 1 | - |
| Compositions for Enhancing Targeted Gene-Editing and Methods of Use Thereof | - | 6 |
| Compositions and Methods for Treatment of Cystic Fibrosis | - | 3 |
| Compositions and Methods for In Utero Delivery | - | 1 |

Furthermore, the Company owns a trademark application no. 87,395,954 filed on April 3, 2017 for “TRUCODE”.[[7]](#footnote-8)

Management Team

The Company has approximately 15 employees. Key members of the Management team are:

**Marshall W. Fordyce, MD – *President &* *Chief Executive Officer (“CEO”)***

Dr. Fordyce is currently CEO of the Company. Previously, he worked at CDF as CEO from July 2016 through May 2017. Prior to that, he worked as Senior Director, Clinical Research for less than a year in 2016, Director, and Clinical Research for two years from 2014 through 2016, and as Associate Director, Clinical Research for three years from 2011 through 2014. He received Doctor of Medicine from the Harvard Medical School and Bachelor of Arts (“B.A.”) from the Harvard University.[[8]](#footnote-9) Dr. Fordyce was previously Senior Director of Clinical Research in HIV Therapeutics at Gilead Sciences, Inc. (“Gilead”), where he led the development of the antiviral drug, tenofovir alafenamide, to replace the cornerstone of therapy for patients with chronic HIV and hepatitis B infections with a safer drug.

Dr. Fordyce has worked with the non-profit organization Partners in Health on health care access in inner-city Boston, and on improving global access to tuberculosis drugs. He completed his Internal Medicine Training at New York University/Bellevue Hospital, where he served as Senior Chief Resident, and completed his Infectious Disease training at Columbia Presbyterian Hospital. Dr. Fordyce was Instructor in Clinical Medicine at The Rockefeller University and Research Fellow at the Aaron Diamond AIDS Research Center, where under David Ho, his research focused on the effect of the HIV entry inhibitor ibalizumab on the envelope protein, and the immunologic effects of initiating antiretroviral therapy during acute infection. Dr. Fordyce is board certified in Internal Medicine and Infectious Diseases and is Volunteer Physician and Medical Director of the RotaCare Coastside Free Clinic in Half Moon Bay, California. Dr. Fordyce became a member of the Board of Directors of the Albert and Mary Lasker Foundation in 2012.[[9]](#footnote-10)

**Allen Ebens, Ph.D. – *Chief Scientific Officer (“CSO”)***

Dr. Ebens has twenty-two years of professional scientific leadership and management experience. He has leadership experience in drug discovery from concept to clinic across multiple therapeutic platforms including antibodies, small molecule drugs, antibody-drug conjugates, and cell-based therapies. Prior to joining the Company, Dr. Ebens was Senior Director of Immune Oncology at NGM Biopharmaceuticals (“NGM”). Prior to NGM, Dr. Ebens was Senior Director of Discovery Research at Juno Therapeutics. Preceding that, he was Director of Research Oncology at Genentech.

Dr. Ebens holds a Ph.D. in Molecular Biology from University of California, Los Angeles and a Bachelor of Science degree in Chemistry from University of Washington.[[10]](#footnote-11)

**James M. Coull, Ph.D. – *Consultant, R&D***

Dr. Coull has extensive experience in R&D, IP creation and strategy, and has built successful life science and diagnostic companies. Prior to joining the Company, Dr. Coull was CEO of PNAi. Prior to PNAi, Dr. Coull served as Chief Technology Officer (“CTO”) of AdvanDx, Inc., which created the first FDA-approved molecular tests for rapid diagnosis of bacterial and fungal bloodstream infections. Preceding that, he served as Vice President (“VP”) of Commercial Operations, and subsequently, CTO of Ensemble Discovery Corporation, a venture-backed pharmaceutical discovery company located in Cambridge, Massachusetts. Dr. Coull also co-founded and led R&D at Boston Probes, Inc. (“Boston Probes”), where he spearheaded the development of the company’s core PNA technology and probe-based tests for life science applications. He has comprehensive knowledge and understanding of PNA-based technologies and applications. Boston Probes was acquired in 2001 by Applied Biosystems (“ABI”) whereupon Dr. Coull served as a Senior R&D Manager at ABI until 2004.

Before founding Boston Probes, Dr. Coull held other senior leadership positions at Millipore Corporation (“Millipore”) and PerSeptive Biosystems, Inc. (“PerSeptive”), where he supervised teams of scientists working on the development of new methods and materials for PNA synthesis and analysis. He also conducted research on DNA/peptide synthesis and protein analysis leading to new product lines at Millipore and PerSeptive. As a pioneer of many innovative techniques, Dr. Coull is listed as an inventor on more than thirty issued US patents, has published over forty peer-reviewed articles, and presented numerous posters and oral presentations at international scientific meetings and conferences. Dr. Coull holds a Ph.D. from Purdue University and a Bachelor of Science degree from Colby College. [[11]](#footnote-12)

**Lauren Frenz – *SVP, Corporate Strategy & Finance***

Ms. Frenz joined the Company as SVP, Corporate Strategy & Finance in July 2017. Previously, she served as Associate Director of HIV Marketing at Gilead, where she led US healthcare provider marketing for GENVOYA, a prescribed HIV regimen with $1.3 billion in annual revenues in its first year of launch. She also led launch planning, brand strategy, messaging, and tactic development for several other drugs. Prior to her role in marketing, she served in the Commercial Planning group in positions of increasing responsibility, leading the development of market insights, long-range forecasting, product positioning, payer value strategies, and global branding in various therapeutic areas. Ms. Frenz has past experience as an Associate at Leerink Partners in the Strategic Advisory group, devising business development, commercial, and portfolio management strategies for biopharma companies.

Ms. Frenz earned her Master of Business Administration at Harvard Business School and an AB, *cum laude*, from Princeton University in Psychology with a Certificate in Neuroscience. Ms. Frenz serves on the Board of the Harvard Business School Healthcare Alumni Association.[[12]](#footnote-13)

Capitalization and Ownership

The Company is authorized to issue common and preferred stock. As of the Valuation Date, the following

shares were issued and outstanding or anticipated to be issued and outstanding:

*Table 4: Capitalization Table*

|  |  |  |  |
| --- | --- | --- | --- |
| EQUITY CLASS | AUTHORIZED | OUTSTANDING | AS CONVERTED |
| **Series Seed** | 1,010,456 | 1,010,456 | 1,010,456 |
| **Series Seed-1** | 1,787,640 | 1,787,640 | 1,787,640 |
| **Series A** | 6,120,111 | 6,120,111 | 6,120,111 |
| **Series B** | 6,989,000 | 5,097,566 | 5,097,566 |
| **Common** | 23,000,000 | 3,618,273 | 3,618,273 |

As of the Valuation Date, the Company had 1,737,874 Common Stock options outstanding and 1,602,601 Common Stock options reserved for future grants as part of Company’s equity-based compensation plan (the “Options Available”). According to Management, 100.0% of the Options Available are expected to be granted in the near future and prior to a possible exit/liquidity event for the Company. As such, due to the dilutive impact of the option pool, we included these shares in our analysis.

Relative to the Previous Valuation, the Company did not meet its expectations to raise the second tranche of Series B (“Series B Tranche II”) (1,891,434 shares that were anticipated to be issued by the end of 2018). As such, the Company reduced its cash burn budget for 2019 and 2020 and focused its research efforts on only one program.

Below is a summary of Common Stock options outstanding as of the Valuation Date:

*Table 5: Common Stock Options Summary*

|  |  |  |  |
| --- | --- | --- | --- |
| CLASS OF STOCK | EXERCISE PRICE | SHARES | |
| **Common Stock Options** | $0.006 | | 10,000 |
| **Common Stock Options** | $0.350 | | 79,688 |
| **Common Stock Options** | $0.380 | | 328,690 |
| **Common Stock Options** | $0.810 | | 1,224,401 |
| **Common Stock Options** | $0.910 | | 95,095 |

Refer to Exhibit G.1 for further details on the Company’s capitalization.

Future Financing

Based on discussions with Management, we considered the timing and magnitude of future rounds of financing that would be necessary for product development. Management estimated that the Company would require the below mentioned additional funding prior to reaching a possible exit:

1. Equity Raise #1: Management expects to raise $50.0 million at the end of the third quarter of 2019. Based on the Company’s expense forecasts, the Company may run out of cash by November 30, 2019 and may require an equity raise to sustain its operations and for IND filing of its lead candidate; and
2. Equity Raise #2: Based on the discussions with Management and the cash burn analysis prepared based on the Company’s expense forecast, RNA assumed $50.0 million equity raise at the end of 2021 for the clinical trials of its lead candidate.

Stage of Development

The Practice Aid defines six stages of development for start-up enterprises.

Stage 2 companies lack product revenue, but have substantive expense history. Product development is underway and business challenges are thought to be understood. Stage 2 companies customarily have second or third round financing. Typical investors are venture capital firms; they may provide additional management or board of directors’ expertise. Generally, securities issued to those investors are in the form of preferred stock.

RNA classifies this Company as a Stage 2 company because it has no product revenue and the Company’s product and technology are at an early stage of development. However, the Company has raised two rounds of preferred financing.

Industry Overview

Overview

In valuing a business or its assets, it is important to consider the condition of, and the outlook for, the industry in which the enterprise operates. Depending upon the nature of the marketplace, industry conditions can significantly affect financial performance and, consequently, value. The following section provides a brief overview of global gene editing market, CRISPR/Cas9 market, and the hemoglobinopathies market (including SCD market) as well as a discussion of the competitive environment in that space.

Genome Editing Market

Genome editing is a type of genetic engineering technique that involves insertions, deletions, replacements or modifications of DNA in the genomes of living organisms.[[13]](#footnote-14) According to a report from Research and Markets, the global genome editing market is anticipated to reach $10.7 billion in 2025 from $3.2 billion in 2018, representing a CAGR of 17.0%. The market growth is driven by the increase in funding for genome editing research, rising prevalence of genetic disorders, increase in the advancements for genome editing technology and the rise in the production of genetically modified crops.[[14]](#footnote-15)

Genetic modification has provided an approach for reverse genetics, analyzing gene function, and linking DNA sequence to phenotype. Traditional methods are now becoming obsolete, due to progress in the genome sequencing technologies, as a result of inefficient, time-consuming, and labor-intensive methods. Recent technologies, like CRISPR/Cas9 nuclease, can initiate genome editing easily and precisely, with little to no limitations by the organism. These instruments offer intriguing possibilities for conducting large-scale experiments. Thus, technological advancements further support the growth of the genome testing market.[[15]](#footnote-16)

The genome editing market, per technology, is segmented as transcription activator-like effector nucleases (“TALENS”), CRISPR, zinc finger nucleases (“ZFNs"), antisense RNA and others. In 2017, CRISPR technology, among the technology segment, held the largest market share of 53.6% and is expected to maintain its dominance during the forecast period of 2018 to 2025. On the other hand, TALENs contributed 23.3% to the market in the year 2017 and is expected to be the fastest growing market in the near future. CRISPR technology is a widely used technology, primarily due to the fact that comparatively, it offers simple, fast and accurate functioning.[[16]](#footnote-17)

Geographically, North America dominated the market in terms of revenue share, due to higher adoption rates for the advanced technology in the research institutes primarily based in the US. Furthermore, the incidence of genetic diseases are high in the US.[[17]](#footnote-18)

CRISPR Technology Market

The global CRISPR technology market is anticipated to reach $5.3 billion by 2025, rising from $1.2 billion in 2017. The global CRISPR technology market is projected to grow at a CAGR of 20.2% from 2018 through 2025, aided by the rising government spending in genome editing, greater incidence of genetic disorders, and the usage of CRISPR/Cas9 technology for improving crop production.

In 2017, the global CRISPR/Cas9 mediated drug discovery and development market was estimated at $435.0 million. The market is expected to jump to $1.3 billion by 2020 and grow at a CAGR of 23.8% over a period of eight years from 2017 to contribute $2.4 billion by 2025. The drug discovery application segment held 35.6% of CRISPR/Cas9 market share in 2017, which is expected to increase steadily over the forecasted period and comprise 45.2% of the total market share in 2025. Within the CRISPR/Cas9 tools market, the clinical therapy segment is expected to contribute significantly. In 2017, the global CRISPR/Cas9 mediated clinical therapy market was estimated at $185.0 million. The market is expected to grow by a CAGR of 19.0% over a period of eight years from 2017 to contribute $744.0 million by 2025.[[18]](#footnote-19)

Hemoglobinopathies and SCD Market

According to a report from Technavio, the global hemoglobinopathies treatment market is predicted to exhibit an incremental growth of $3.2 billion during the period 2019 to 2023, registering a CAGR of 10.5%. The market growth is supported by lack of approved therapies, entry of several pipeline products in late stage, and approval of currently late stage pipeline products.[[19]](#footnote-20) The market is subject to witness a substantial growth due to the growing prevalence of hemoglobinopathies, particularly in underdeveloped economies such as South East Asia, the Middle East region, and Africa. In addition, the increasing number of high unmet needs for diagnosis and treatment of these disorders is expected to fuel the growth of hemoglobinopathies market over the next seven years. Globally, the market is predicted to grow at a higher CAGR during the forecast period of 2018 through 2022, providing numerous opportunities for market players to invest for R&D in the market. Rising mortality rate, particularly diseases like SCD and thalassemia are expected to generate demand for this market. Additionally, favorable government laws and regulations coupled with growing investment by local governments are estimated to stimulate the growth of the market. A substantial focus has been given to spreading awareness about diseases like SCD and thalassemia, and development of Hemoglobinopathies (“RuSH”) are predicted to boost the market growth over the period of 2018 through 2022.[[20]](#footnote-21)The global SCD treatment market is expected to reach $5.5 billion by 2023, exhibiting a 14.3% CAGR during the forecast period of 2018 through 2023. Several factors such as an increase in investments for R&D, growing target population, and favorable government initiatives are anticipated to drive the market. High unmet medical needs, strong pipeline, and growing patient pool are key factors expected to influence market growth. [[21]](#footnote-22) The global SCD treatment market has witnessed a significant increase in the number of SCD cases in recent years. According to the Centre for Disease Control and Prevention, approximately 100,000 Americans were afflicted by SCD in 2017. Currently, there are no approved therapies for the treatment of SCD. At present, the market has only three approved therapies for SCD symptoms, and six molecules are in Phase III of clinical trials.[[22]](#footnote-23) In recent years, the demand for cost-effective drugs and gene therapies for SCD has increased owing to unavailability of a permanent cure and risks associated with a bone marrow transplant. Currently, 20 to 25 million people worldwide are reportedly living with SCD and about 300,000 infants are born annually with this disease. The US dominated the global market and is projected to maintain its position through 2023, owing to a rise in the incidence of disease and the launch of promising pipeline candidates.[[23]](#footnote-24)

The therapies approved currently for SCD only provide symptomatic relief, and no cure exists for the disease yet. Some of the FDA-approved medications for SCD treatment are:[[24]](#footnote-25)

1. Hydroxyrea – Originally approved in 1998, for adults only, it helps red blood cells to stay round and flexible, which can help reduce complications;
2. Endari (L-glutamine oral powder) – Approved in July 2017, to reduce acute complications of SCD, including the frequency of sudden, severe attacks of pain. It is cleared for patients aged 5 years and older and was the first new treatment in nearly 20 years; and
3. Hydroxyurea - Approved in December 2017, for patients aged 2 years and above, for reducing the frequency of painful crises and the need for blood transfusions.

Apart from the above, blood transfusions for treating anemia (a common complication of SCD), is another symptomatic treatment. In terms of surgical treatments, bone marrow or stem cell transplant may be an option for younger patients, but they present serious and life-threatening side effects. Also, transplants require a matching bone marrow or stem cell donor, which can be difficult. Thus, making transplant an unviable option in most cases.

Competition

The Company faces competition from other companies in the genome editing and SCD treatment industry. Some of the key competitors include Fibrocell Science, Inc., Emmaus Life Sciences, Inc., Modus Therapeutics AB, Global Blood Therapeutics Inc., bluebird bio, Inc., Sangamo Therapeutics, Inc., Editas Medicine, Inc., Intellia Therapeutics Inc. and CRISPR Therapeutics AG.[[25]](#footnote-26)

Valuation Methodology Overview

The following section provides an overview of various valuation methodologies considered as part of this analysis.

Business Enterprise Valuation Theory

In summary, there are three generally accepted valuation approaches available when valuing the operating assets and liabilities of a closely held business:

1. Cost Approach (“Cost Approach”);
2. Income Approach (“Income Approach”); and
3. Market Approach (“Market Approach”).

Within each category, a variety of methodologies exists to assist in the estimate of value. They are discussed in further detail herein. In addition, there is the Hybrid Approach (“Hybrid Approach”), a methodology that combines two or more of these approaches.

Cost Approach

The Cost Approach relies upon separately valuing each sub-component of the company being valued. The discrete valuation of an asset using this approach is based upon the concept of replication or replacement as an indicator of value. In essence, this method answers the build approach when looking at a “buy versus build” approach to investment.

In the case of most IP-centric technologies with explicit patent protection and substantive and broad blocking rights to competitive entrants, the Cost Approach is not a reasonable proxy for value. By the time most products are commercial, the cost to recreate the existing asset is prohibitive in these circumstances given the demanding regulatory requirements. Furthermore, these costs should be considered sunk costs and, as such, other approaches to value should be considered.

Income Approach

The Income Approach is based on the earnings power, or the cash generating abilities of the company being valued. This approach focuses on determining a forecast benefit stream that is reflective of the subject company’s most likely future performance. The forecast benefit stream is then discounted to present value based on the appropriate risk‐adjusted discount rate or capitalization rate. The DCF is a commonly used Income Approach. In addition, in the life sciences, if clinical or regulatory risks remain, an rNPV is also common. For IP-centric rights interests, with reasonable patent protection and expectations for a significant decrease in market share upon patent expiration, the Income Approach is generally the favored approach by industry professionals.

Market Approach

In summary, the Market Approach references actual transactions involving (i) the subject being valued, or (ii) similar assets and/or enterprises. The Market Approach generally consists of two primary methodologies: the Guideline Comparables Method (“GCM”) and the Guideline Transaction Method (“GTM”). The GCM involves identifying and selecting publicly traded companies or guideline public companies (“Guideline Public Companies”) with financial and operating characteristics similar to the subject being valued, and subsequently deriving multiples and other metrics from such Guideline Public Companies to apply to the subject being valued. The GTM involves identifying and selecting actual transactions, such as mergers, acquisitions, investments, and licensing agreements, involving companies and/or assets with financial and operating characteristics similar to the subject being valued, and subsequently deriving multiples and other metrics from such guideline transactions (“Guideline Transactions”) to apply to the subject being valued.

The third method under the Market Approach that can provide an indication of value is the Option Pricing Model Backsolve Method (the “OPM Backsolve Method”). By considering the sale price of shares in a recent financing, the equity value can be “back-solved” using an option pricing model that gives consideration to the Company’s capitalization structure and the rights of the preferred and common shareholders. This methodology is most applicable when a valuation is conducted close to the date of a financing transaction, and when other methodologies are deemed less reliable.

Hybrid Approach

A Hybrid Approach combines two or more of the approaches above. Typically, this involves some combination of an Income Approach with a Market Approach. For example, a licensing transaction, which has definitive upfront payments, milestones and royalties, can be viewed as an Income Approach; however, the determination of reasonable consideration across the three major approaches can be based on comparable license arrangements for similar assets which can be viewed as a Market Approach.

Allocation Methodology Theory

In summary, there are four generally accepted allocation methodologies available when determining the value of various classes of securities underlying a company’s capital structure:

1. Current Value Method;
2. Option Pricing Method;
3. Probability-Weighted Expected Return Method; and
4. Hybrid Method.

Within each category, a variety of methodologies exists to assist in the estimation of value, as discussed in further detail herein.

Current Value Method (“CVM”)

The CVM is based on an allocation theory that shareholders with senior stock rights would attempt to maximize the value of their holdings based solely on the senior interest’s underlying liquidation preference, participation rights and conversion rights, as well as an imminent liquidity event. In essence, this approach determines the value of the enterprise at the Valuation Date, distributes said value through the existing capital structure waterfall and then applies discounts or premiums as may be appropriate to the varying security classes. It does not consider optionality or upside payoffs for those securities that may not receive value at the current valuation (e.g. value does not exceed preference) but may receive value if value increases over time (e.g. future value exceeds preference).

RNA noted that the CVM is appropriate under following circumstances:

1. When an imminent liquidity event in the form of an acquisition or dissolution of the enterprise is assumed and/or the expectations about the future of the enterprise as a going concern are effectively immaterial; and
2. When the enterprise is assumed to be at such an early stage of its development that:
   1. No material progress has been made on its business plan;
   2. No significant equity value has been created above the liquidation preference of the preferred stock; and
   3. There is no reasonable basis for estimating the timing and magnitude of any common equity value above the liquidation preference that might be created in the future.

Option Pricing Method (“OPM”)

The OPM relies on financial option theory to allocate value among different classes of members’ equity based upon a future option “claim” on value. Under the OPM, the values of the various classes of stock are estimated as the net value of a series of call options, representing the present value of the expected future returns to the shareholders.

Essentially, the equity claims of a shareholder class are equivalent to a call option on the stock’s participation in the value of the subject company at or above the respective preferred shareholders’ liquidation preferences. Thus, an equity class can be valued by estimating the value of its share in each of these call option rights.

The OPM involves estimating the value of the call options using the Black-Scholes option pricing model (“Black-Scholes”)[[26]](#footnote-27) a lattice model (“Lattice”)[[27]](#footnote-28) or a risk-neutral Monte Carlo simulation at a series of exercise prices that coincide with the liquidation and conversion preferences of the holders of preferred and common shareholders. The Black-Scholes model and most Lattice models assume that a company’s or an asset’s potential return distribution follow a log-normal path and that the period to period movement in price follow a geometric Brownian motion (meaning that prices fluctuate smoothly from period to period and do not essentially jump). Furthermore, closed form models like Black-Scholes do not allow for certain types of liquidity events, like structured sales, which may have some contingent consideration component as part of the exit value. Simulation techniques can capture the reality of most assets noting that companies and asset values usually “jump” on data, that returns can be bi-modal for development stage assets, and that a license or structured exit on the back-end can be simulated. That said, simulation techniques can be costly to develop, can have limited transparency, and can be understood by a much smaller audience than the other techniques.

RNA also noted that the OPM may be used to determine the equity value of a company by using the OPM Backsolve Method. In an OPM framework, the equity value is inferred from a recent financing transaction. It involves making assumptions for the time to liquidity, volatility, and risk-free rate and then solving for the value of equity such that value for the most recent financing equals the amount paid.

In general, while simple in its application, especially for Black Scholes OPM techniques, RNA does not typically apply these approaches when considering allocation techniques for the valuation of equity interests in early stage, privately held life science companies. In doing so, we would violate the major assumptions of both the Black Scholes and the Lattice approaches. Additionally, the simulation approach can generally be reasonably approximated by a scenario-based approach like the PWERM as described below.

Probability-Weighted Expected Return Method (“PWERM”)

Under the PWERM, the value of a company’s particular equity class is estimated based upon an analysis of future values for the entire enterprise assuming various future outcomes. Share value is based upon the probability-weighted present value of these expected outcomes, as well as the rights of each class of preferred and common stock.

The PWERM is well suited for capturing potentially dramatic increases or decreases in value that may result from potential future events that are not log-normally distributed that have the potential for structured exits with contingent consideration. It can take into account elements that apply when considering real-world, risk-adjusted decision frameworks. Candidly, it is the framework most business development, private equity and other transactional professionals consider when thinking through the potential outcomes for an enterprise (i.e. a decision tree or a scenario analysis). We noted that the application of the PWERM is reasonable under circumstances where there is a broad range of possible future outcomes for the enterprise noting that the likelihood of such outcomes and the resulting valuation indications are not assumed to be log-normally distributed as under the OPM. RNA also noted that the PWERM may be used to determine the enterprise value of a company given the contemplation of future values for the entire enterprise assuming various future outcomes in a back-solve type of methodology as is noted above for the OPM, albeit a bit more complex with many more inputs to balance. For privately-held companies with complex capital structures in the life sciences, it is generally RNA’s preferred approach to use a PWERM construct to allocate value across the various security classes.

Hybrid Method

The Hybrid Method is a cross between the PWERM and OPM. It is performed by first estimating the probability-weighted value across multiple scenarios then use the OPM to estimate the allocation of value within one or more of those scenarios. The Hybrid Method can be a useful alternative to explicitly modeling all PWERM scenarios in situations when the company has transparency into one or more near-term exits but is unsure about what will occur if the current plans fall through.

An advantage of this method is that it utilizes the conceptual framework of option pricing theory to model a continuous distribution of future outcomes and to capture the option-like payoffs of the various share classes while also explicitly considering future scenarios and the discontinuities in outcomes that early-stage companies experience. A disadvantage is that these models require a number of assumptions and may be overly complex. In addition, there may be many potential issues surrounding the integration of risk-adjusted and risk-neutral modeling frameworks.

Valuation Analysis

Selected Valuation Approaches

Considering the above, the valuation determination herein has been developed primarily on the basis of the PWERM. Taking the stage of development of the Company and expected liquidity events into account, we elected not to rely upon a pure application of the three general valuation approaches discussed above. Notwithstanding, we did consider elements of the Market and Income Approaches for gauging the appropriateness of certain PWERM inputs and assumptions. Refer to Exhibit B.1 for details on the PWERM.

Income Approach

The DCF method aggregates the present value of all future cash flows available to the investment holder to determine the valuation indication as of the Valuation Date. The DCF methodology involves the following key steps:

1. Determination of cash flow forecasts (“Representative Level Projections”); and
2. Selection of a range of comparative investment risk-adjusted discount rates to apply against the Representative Level Projections.

For purposes of this analysis, as detailed above, RNA did not rely on the DCF in determining a valuation indication for the Company. Notwithstanding, RNA did consider discount rates, a key component of the PWERM.

**Discount Rate**

A discount rate represents the rate of return an investor requires to justify investment in a company while giving consideration to the risk associated with the investment. Discount rates are determined based on market expectations of the total rate of return and the rate at which capital will be attracted to a company. One of the most important considerations in determining an appropriate discount rate is the level of risk inherent within a company. Therefore, due consideration is given to the rates of return available on alternative, comparable investments available to a hypothetical buyer.

Numerous factors influence the choice of an appropriate discount rate including those factors external (potentially systematic) and internal (potentially unsystematic) to the potential investment. External factors include, but are not limited to, (i) current general economic conditions, (ii) expectations regarding future economic conditions as of the analysis date, (iii) sources of capital available to a company and (iv) competitiveness of the markets served by the company. Internal factors include but are not limited to (i) the financial situation of the Company, (ii) the ability to generate positive cash flows, (iii) the likelihood of the Company facing difficulty in procuring raw inputs and (iv) the ability to deliver products to an available market.[[28]](#footnote-29)

Three studies in the Practice Aid outline estimated return requirements for companies at various stages of development.

*Table 6: Required Rates of Return by Stage of Development*

|  |  |  |  |
| --- | --- | --- | --- |
| **RATE OF RETURN ANALYSIS** | | | |
| **Stage of Development** | **Plummer** | **Scherlis and Sahlman** | **Sahlman and Others** |
| **Start Up** | 50% to 70% | 50% to 70% | 50% to 100% |
| **Early Development** | 40% to 60% | 40% to 60% | 40% to 60% |
| **Expansion** | 35% to 50% | 30% to 50% | 30% to 40% |
| **Bridge / IPO** | 25% to 35% | 20% to 35% | 20% to 30% |

Source: AICPA Valuation Guide, Appendix B (Venture Capital Rates of Return)

The Practice Aid task force defined each stage of development as the following:

**Start-Up (“Start-Up”):** Start-up-stage investments are typically made in enterprises that are less than a year old. The venture funding is to be used substantially for product development, prototype testing, and test marketing.

**Early Development (“Early Development”):** Early-development-stage investments are typically made in enterprises that have developed prototypes that appear viable and for which further technical risk is deemed minimal, although commercial risk may be significant.

**Expansion (“Expansion”):** Enterprises in the expansion stage usually have shipped some product to consumers (including beta versions).

**Bridge/IPO (“Bridge/IPO”):** Bridge/IPO-stage financing covers such activities as pilot plant construction, production design, and production testing, as well as bridge financing in anticipation of a later initial public offering.

Notwithstanding, these rates of return are not inclusive of the probabilities associated with achieving such returns. As such, RNA considered the probabilities of success (as contemplated under the PWERM) in determining the appropriate cost of capital for the Company’s different classes of securities, as discussed below.

For the purposes of determining discount rates under the PWERM, RNA noted the following:

1. **Preferred:**
2. The preferred portion of the Company’s capital structure was bifurcated into debt-like and equity-like components. The debt-like piece reflects liquidation preferences (or the equivalent value to the extent preferred converts) while the equity-like segment reflects value over and above such liquidation preferences;
3. The values for both were calculated as the probability-weighted present value of each relevant component under the PWERM;
4. The cost of capital for the debt-like portion was based on rates for venture debt based on RNA’s observations of such markets; and
5. The cost of capital for the equity-like component was a back solve in order to achieve approximately 15.0% to 20.0% VC portfolio returns (across the total preferred investment). Such VC portfolio returns are generally consistent with observations of historical and expected VC returns for early stage biotechnology investing (adjusted for carried interest).

1. **Equity:**
2. The share count included outstanding Common Stock with consideration of issued warrants, options and other securities, as appropriate;
3. The price was generally in line with the concluded fair market value of the Common Stock; and
4. The rate reflects consideration of an implied return to common (based on the probability of successful exits under the PWERM) higher than the implied return to preferred stock investors due to the higher risk profile of common Stock relative to preferred stock. More specifically, we noted that the common stock holders are entrepreneurs and entrepreneurial employees and are not typically well-diversified investors. Based on the article “Opportunity Cost of Capital for Venture Capital Investors and Entrepreneurs” by Kerins, Smith and Smith, entrepreneurs have a 2.0x to 4.0x cost of capital differential from investors. However, since we have applied an additional adjustment risk factor (as explained in detail in the latter part of the Report), we elected to apply a lower discount rate, which is in line with the cost of capital for the equity-like component of the Company’s preferred stock. These returns indicate a marketable level of value and do not include a DLOM, so we explicitly considered the application of DLOM (explained in detail in the latter part of the Report) for the PWERM scenarios to estimate the non-marketable price of the Common Stock.

Because private enterprises like the Company often seek financing from private equity investors, including VC firms, the VC arena provides an observable market for the cost of capital for privately held enterprises. The following table illustrates the dollar weighted internal rate of return on vintage year investments in the life sciences by VC firms, as published by Cambridge Associates and the National Venture Capital Association (“NVCA”):

*Table 7: Venture Capital Weighted IRR*

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| **VENTURE CAPITAL DOLLAR WEIGHTED INTERNAL RATE** | | | | | | | | | | | | | |
| **OF RETURN ON VINTAGE YEAR COMPANIES** | | | | | | | | | | | | | |
| **Industry** | **2005** | **2006** | **2007** | **2008** | **2009** | **2010** | **2011** | **2012** | **2013** | **2014** | **2015** | **2016** |
| Biotechnology/ Biopharma/R&D | 14.81 | 7.25 | 17.79 | 11.47 | 27.05 | 30.07 | 36.96 | 60.58 | 98.81 | 51.77 | 48.73 | 44.34 |
| Healthcare Devices | 8.94 | 2.88 | 4.62 | 4.19 | 0.99 | 11.78 | 15.04 | 13.42 | 1.78 | 5.14 | 25.88 | 24.76 |
| Healthcare Services | 12.31 | 6.34 | 17.04 | 7.48 | 26.17 | 6.51 | 26.31 | 28.94 | 41.74 | 28.44 | 28.46 | 18.15 |
| Healthcare Software/Systems | 9.18 | 7.11 | 17.01 | 49.45 | 7.76 | 12.97 | 30.55 | 9.77 | 9.66 | 18.37 | 22.02 | 12.89 |
| Pharmaceuticals | 4.12 | 5.74 | 21.74 | 9.42 | 26.68 | 30.12 | 14.76 | 63.78 | 21.27 | 39.04 | 26.12 | 26.5 |

*Source: Cambridge Associates LLC US Venture Capital Index® and Selected Benchmark Statistics, September 30, 2018*

These rates of return illustrate the pooled gross IRR to investors in VC funds. The returns on investments in the biotechnology and pharmaceutical sectors have been strong since 2010; however, over a longer duration (and a full business cycle), return expectations would be significantly less than recent performance and expected return thresholds would be more consistent with an average historical performance range of 15.0% to 25.0%, especially if the industry sectors go out of favor.

Based on the considerations herein, RNA estimated an enterprise-level WACC of 20.0% as the discount rate, which has been calculated as a blended rate of all the components of capital structure. Refer to Exhibit D.1 for further details.

Market Approach – GCM

The GCM involves the determination of a valuation indication by multiplying a representative level of earnings, cash flows or other measure against an appropriate risk‐adjusted multiple. This approach provides an indication of value for a company or asset that corresponds with the particular earnings figure being capitalized on a controlling or non‐controlling basis dependent on the underlying levels of multiples applied.

For purposes of this analysis, RNA did not rely upon the GCM in determining the enterprise value of the Company since it is a pre-revenue company. Notwithstanding, RNA did consider Guideline Public Companies deemed comparable to the Company, a key element of the Market Approach. Refer to Exhibits E.1 and E.2 for details on enterprise value, projections, operating metrics, market capitalization, business descriptions, and other metrics for the selected Guideline Public Companies.

For purposes of this analysis, RNA considered the following Guideline Public Companies under the categories mentioned below:

**Blood Disorders/Gene Editing/Gene Therapy:**

1. Alnylam Pharmaceuticals, Inc.;
2. bluebird bio, Inc.;
3. Ironwood Pharmaceuticals, Inc.;
4. Global Blood Therapeutics, Inc.;
5. Acceleron Pharma Inc.;
6. Spark Therapeutics, Inc.;
7. REGENXBIO Inc.;
8. uniQure N.V.;
9. Audentes Therapeutics, Inc.;
10. Sangamo Therapeutics, Inc.;
11. Homology Medicines, Inc.;
12. Corbus Pharmaceuticals Holdings, Inc.;
13. GlycoMimetics, Inc.;
14. LogicBio Therapeutics, Inc.;
15. Arbutus Biopharma Corporation;
16. Voyager Therapeutics, Inc.;
17. La Jolla Pharmaceutical Company;
18. Protagonist Therapeutics, Inc.; and
19. Fibrocell Science, Inc.

**CRISPR/Cas9:**

1. CRISPR Therapeutics AG;
2. Editas Medicine, Inc.;
3. Intellia Therapeutics, Inc.; and
4. Abeona Therapeutics Inc.

RNA acknowledged the characteristics of the Company that differ from the characteristics of the Guideline Public Companies, detailed as follows:

1. The Company's operations focus primarily on developing therapies for SCD using its gene editing platform, which is generally different from and/or less diversified than the businesses of the selected Guideline Public Companies;
2. The Company must raise financing in order to develop certain assets (noting that the Guideline Public Companies generally have easier access to capital to develop their programs);
3. The Company is generally smaller and at an earlier development stage than the Guideline Public Companies;
4. The range of WACC observed for the Guideline Public Companies represent the growth and risk profile associated with each of the selected Guideline Public Companies. In general, the strategies and prospects of the Company represent a higher risk profile than the Guideline Public Companies; and
5. The Company is not a publicly traded company and relies on private sources of equity. Public companies typically have lower costs of equity since the public equity markets typically demand lower levels of return compared to private sources of equity. Investments in public companies provide a liquid investment that may compensate for the minority level interest typically involved.

As noted above, RNA did not develop an enterprise value indication based on the application of market multiples.

Market Approach – GTM

RNA did not rely upon the GTM because the Company is pre-revenue. Furthermore, it is relatively difficult to identify market transactions that are reasonably similar to the Company with respect to stage of development, underlying economic fundamentals, products (i.e., potential product pipeline) and prospects for success. Notwithstanding, we did consider Guideline Transactions deemed comparable to the Company, which is a key element of the Market Approach, for purposes of assessing exit values under the PWERM.

RNA reviewed the M&A transactions in recent years in similar industries and considered various transactions that were comparable to the Company. RNA acknowledged the characteristics of the target companies underlying the Guideline Transactions, detailed as follows:

1. The Company’s operations focus primarily on the developing therapeutics for SCD using its gene editing platform, which are generally different from and/or less diversified than the businesses of the target companies;
2. The Company is generally smaller in size than the target companies underlying the Guideline Transactions, and as such, does not benefit from the leverage over suppliers and customers and certain economies of scale to which the target companies may be privileged; and
3. The strategies and prospects of the Company generally represent a higher risk profile relative to most of the target companies since the Company is currently at an earlier stage/smaller than when some of the target companies were acquired.

As noted above, RNA did not develop an enterprise value indication based on the application of market multiples.

Allocation Analysis

Considering the above, the allocation determination herein has been developed primarily on the basis of the PWERM to allocate the equity value of the Company across the Company’s capital structure. Though presented here, we did not rely on the OPM as our primary allocation method given difficulties in the application of the approach due to the disconnect with return distributions required for closed form option models like the Black-Scholes option model. We relied on the PWERM as our primary and only allocation methodology as we believe this methodology is better suited to addressing the outcomes associated with the Company and is better aligned with the methodologies employed by investors in companies similar to the Company.

Current Value Method

Based on the considerations detailed above, RNA noted that since the Company is not expecting an imminent liquidity event and is not at a very early stage of development, circumstances do not meet the appropriate criteria for the application of the CVM. As such, RNA has elected not to rely upon the CVM as a primary allocation methodology.

Option Pricing Method

Based on the considerations detailed above, as well as RNA’s observations of Guideline Public Companies, Guideline Transactions and general industry experience, RNA noted that the possible future outcomes and the resulting enterprise value indications for the Company would not generally follow a log-normal distribution. Given the Company’s early stage of development and limited historical performance and near-term projected performance, the underlying presumptions of a log-normal distribution of returns and a return distribution that follows a Geometric Brownian motion were not reasonable to apply in this analysis. As such, RNA has elected not to rely upon the OPM.

Probability Weighted Expected Return Method

Based on the considerations detailed above, as well as discussions with Management, RNA noted there is a broad range of possible future outcomes for the Company. As such, for the purposes of this analysis, RNA elected to use the PWERM as a primary methodology.

**Exit Modeling**

Management indicated that there may be potential exit opportunities. The projected exits were structured and non-structured M&A and IPO events that may occur after the successful completion of Phase I and Phase II clinical trials of their lead candidate.

The exit scenarios forecasted as part of the PWERM were as follows:

1. **IPO:** The IPO scenarios reflect an exit or liquidity event by means of a sale of stock by the Company to the public. RNA considered an IPO exit at the end of 2020 if the lead candidate successfully completes Phase I trials and at the end of the third quarter of 2022 if the lead candidate successfully completes Phase II trials. The timing was based on Management’s expectations;
2. **M&A:** The M&A scenarios reflect a potential exit for the Company via a merger or acquisition. The M&A exits were modeled in line with the IPO exits mentioned above, based on Management’s expectations;
3. **Low Sale:** The Low Sale scenarios reflect as exit assuming the Company is unable to get an IND approval and initiate the clinical trials or the Company fails to successfully complete the clinical trials, and has difficulty raising additional investor financing. Following are the Low Sale scenarios assumed at different stages of product development:
   1. Low Sale – IND: A Low Sale scenario is assumed at the end of the third quarter of 2019 if the lead product fails to receive an IND approval;
   2. Low Sale – Phase I: A Low Sale scenario is assumed at the end of 2020 if the lead product fails to complete Phase I clinical trials; and
   3. Low Sale – Phase II: A Low Sale scenario is assumed at the end of the third quarter of 2022 if the lead product fails to complete Phase II clinical trials.

Refer to Exhibits B.1 and B.2 for details of the scenarios identified and descriptions of the outcomes forecast in each discrete scenario.

Relative to the Previous Valuation, we presented the exit scenarios based on the development of only one product focused on SCD treatment, compared to development of two products focused on SCD and cystic fibrosis respectively. In line with the Previous Valuation, we combined the Low Sale scenarios to present a single scenario and calculated the probability-weighted exit multiple.

**Structured Exits**

A structured exit coupled with different liquidation preferences creates a framework whereby preferred shareholders may have an accelerated timeline to cash flows when compared to common shareholders. The structure of such a transaction would mean that cash flows to a preferred shareholder and a common shareholder would accrue over a period, and the midpoint of such a period would be 1.0 year from the date of exit. Refer to the “Discounts and Premiums” section below for further details of application of this 1.0 year term.

**Non-Structured Exits (Full Payment on Exit)**

Non-structured exits assume that all exit proceeds are paid on the date of exit. Unlike structured exits, there is no contingent consideration modeled in this type of exit. The structure of such a transaction would mean that cash flows to a preferred shareholder and common shareholder would accrue at the same point in time (i.e., on the date of exit).

IPO and Low Sale exits were modeled as non-structured exits for the purposes of this analysis. M&A exits were modeled as structured exits based on the recent trends in the industry.

**Exit Values**

The exit values were based on discussions with Management, review of GCM and GTM information, review of recent IPO information, licensing deals and RNA’s best estimates.

In order to determine the exit values under the IPO scenarios, RNA considered the valuation of biopharmaceutical companies that went public between 2013 and the Valuation Date. The exit values selected for the IPO Phase I and Phase II are between the first quartile and the mean of the overall range of pre-IPO equity values of recent IPOs of biopharma companies. Based on IPO transactions of companies in Phase I stage of product development, the selected Phase I exit value is close to the first quartile of the range. For Phase II IPO exit, the selected value is close to the mean of the range of IPOs with product development in Phase II stage. Refer to Exhibit H.10 for further details.

In order to determine the exit values for the M&A Phase I scenario, RNA considered the exit value between the first quartile and the median of the overall range of deal values of comparable licensing transactions. For the M&A Phase II scenario, the selected exit value was between the median and mean of the overall range of deal values of comparable licensing transactions. Furthermore, RNA noted that the exit values for all the M&A scenarios were in the range of the median and mean of the transaction values of comparable M&A transactions, however we primarily relied on the licensing deals to determine the exit values. Refer to Exhibit H.9 for further details.

The Low Sale scenarios reflect values resembling negative outcomes and significant sub-1.0x returns to investors, which is consistent with our observations of failed VC investments. The exit value in Low Sale scenarios reflects the value of the Company’s IP and product pipeline at the time of exit. We assumed 0.30x multiple for the Low Sale IND scenario, and 0.50x multiple for the Low Sale Phase I and Phase II scenarios respectively, and calculated the probability-weighted multiple of all the Low Sale scenarios at 0.39x.

Relative to the Previous Valuation, given the exits were assumed based on one-product development compared to two-product development, we reduced the exit values by approximately $50.0 million to $75.0 million. Furthermore, the Company also reduced its cash burn forecast significantly and shut its Woburn operations. In light of these factors, the exit value selections are reasonable.

**Future Financing**

Based on discussions with Management, we considered the timing and magnitude of the future rounds of financing that would be necessary to reach the assumed exits as discussed above.

**Probability Weighting**

Probability weightings and the probability of technical success for clinical phases were established based on discussions with Management and published studies on hematology and blood disorder success rates. For the purposes of this analysis, we considered the Low Sale as the most likely exit scenario, based on the current product development stage and discussions with the Management.

The selected probabilities reflect Management’s anticipation about future exits. Refer to Exhibits B.2 and B.3 for further details. The probabilities assigned to each event are listed below:

1. IPO – Phase I: 1.7%;
2. IPO – Phase II: 1.5%;
3. M&A – Phase I: 14.9%;
4. M&A – Phase II: 5.9%; and
5. Low Sale – Weighted-Average: 76.1%.

**DLOM**

RNA explicitly applied a DLOM since the discount rate applied to the Common Stock include value of the Common Stock on marketable basis. Refer to section “Discounts and Premium” below for further details.

Discounts and Premiums

Discount for Lack of Marketability

The holder of a nonmarketable investment is subject to the risk that the investment’s value will decline before the investment can be sold to another investor in a private transaction. Conversely, the holder of an investment that is identical but for the fact that there exists an active public market is not subject to the same risk. Therefore, the holder of the nonmarketable investment will have a higher required rate of return on the investment than the holder of the marketable investment. Consequently, the nonmarketable investment will sell at a discount to the marketable investment. RNA determined a DLOM as a necessary consideration in the analysis since the minority common stock holders of the Company, unlike the shareholders of the Guideline Public Companies, do not have access to an active public market for their securities. Further, the Company’s common stock holders do not possess the rights to force the Company to register with the Securities and Exchange Commission in order to sell their shares. The lack of marketability of common stock may materially limit a shareholder’s ability to liquidate the investment into cash without the risk of loss in value.

Factors that impact the size of the DLOM fall into two categories: (1) factors that affect the duration of the holding period necessary to locate a buyer and negotiate a sale, and (2) factors that affect the degree of risk faced per unit of time during this holding period. Risk per unit of time, according to modern investment theory, is the volatility of an investment’s total return (i.e., both dividends and capital appreciation), or the propensity for an investment’s actual return to differ from its expected return. Factors that either increase the duration of the holding period or increase the expected volatility of an investment’s total return result in higher DLOM. These factors and their directional impact (all else equal) on the size of the DLOM include the following, among others:

1. Presence/absence of a public market for the investment interest: Presence of a public market eliminates the discount;
2. Availability of information on the underlying business and its financial condition: More information reduces the discount because it is easier for prospective investors to perform valuation analyses of the investment;
3. Complexity of the underlying business and business strategy: Greater complexity increases the discount by making it more difficult for prospective investors to evaluate the business’ opportunities and threats, thus making it more difficult to perform valuation analyses of the investment;
4. Access to Management of the underlying business: Greater access to Management reduces the discount by making it easier for prospective investors to understand the business and evaluate the talent of the business’s management;
5. Information tracking by security/business analysts: Some large private companies release financial and other data and are followed by analysts tracking their publicly traded competitors. The presence of such a following reduces the discount by making it easier for prospective investors to perform valuation analyses of the investment;
6. Potential for a sale of the business or an IPO of stock: Such potential scenarios decrease the discount by reducing the expected total costs to an investor exiting the investment and decreasing the expected time until exit;
7. Variability of the business’s operating profit: Greater variability increases the discount since it increases the degree of risk per unit of time during the holding period (by affecting the outlook for future dividends and also, therefore, the valuation of the investment);
8. Financial leverage in the business: Higher leverage increases the discount since it increases the degree of risk per unit of time during the holding period (by affecting the outlook for future dividends and also, therefore, the valuation of the investment);
9. Size of the business as measured by sales or total assets: Small size, which has been associated empirically with greater overall business risk, increases the discount and vice versa; and
10. Regularity of distributions to equity holders: The expectation of regular future distributions monetized for equity holders in a flow through company and the potential for insufficient cash distributions to satisfy their tax liabilities (attributable to such company’s income) increase the discount.

With respect to a DLOM, empirical observations exist that show that, when investors consider alternative investments in either a freely traded security or a security whose marketability is limited, they will price the security with limited marketability at a discount to its freely traded counterpart. Market evidence of the DLOM can be found in two types of transactions, among others: (1) private placements of restricted stock by public companies, and (2) private placements of stock by private companies that later undergo initial public offerings of their stock.

**Finnerty Analysis**

In particular, John Finnerty proposed a model that assumes the investor does not possess special market timing ability and would be equally likely to exercise the hypothetical liquid security at any given point of time. The value of marketability was modeled as the present value of cash flows, similar to an average-strike put option. The Finnerty method addresses the issue of assuming perfect market timing in Longstaff’s look-back option method and the issue of assuming protection on the downside while still realizing appreciation on the upside in the protective put method. Finnerty also performed a regression analysis to restricted stock studies, adjusting to remove other significant factors, such as concentration of ownership and information effects, and found that after isolating the marketability-related factors, the discounts predicted by his method are consistent with the data. Finnerty presented an updated version of his model at the American Society of Appraisers’ Advanced Business Valuation conference in October 2009.

**Asian Put Analysis**

The Finnerty average-strike put option model, also called an Asian put option, assumes that the put option is struck at the average price of the stock over the period from valuation date to expiration date. The seller is not assumed to have any special market timing ability.

**DLOM Conclusion**

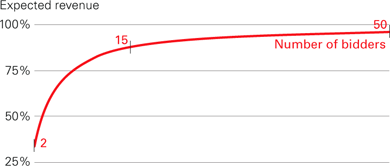
As noted above, in the “Structured Exits” section, cash flows to a preferred shareholder and a common shareholder accrue over a period in a structured exit for a biopharma company. As such, to reflect the additional term to cash flows to common shareholders, we added a 1-year term to exit in the DLOM calculations for the M&A scenarios. Based on the quantitative and qualitative analysis above, RNA concluded a DLOM of 30.0% and 40.0% for the Phase I and Phase II IPO exits respectively and 34.5% and 40.0% for Phase I and Phase II M&A exits respectively. Refer to Exhibit H.1 for further details.

**Non-Diversification Risk Factor**

RNA applied an additional adjustment factor of 30.0% to account for the non-diversification risk of a key group of potential transaction participants and for the nature of the pricing adjustments given the bidding dynamics that would expect to take place in a transaction in the common stock. The nature of a transaction in the common stock must contain the perspectives of those types of investors/holders that would buy and sell in the security. In general, this means that there are two groups that invest in/own rights to these securities. The first group is those that actually hold the securities and these are typically the entrepreneurs and employees that are provided these securities as offsets for other lower compensation arrangements frequently associated with joining early-stage or startup businesses. The second group is investors that might better represent diversified financial investors. Realistically, a fair market valuation standard for a transaction for these securities would need to incorporate the viewpoints of both classes of securities holders.

In general, common stockholders warrant a different rate of return compared to preferred stockholders. More specifically, we noted that the common stockholders are entrepreneurs and entrepreneurial employees and are not typically well-diversified investors. Based on the article “Opportunity Cost of Capital for Venture Capital Investors and Entrepreneurs” by Kerins, Smith and Smith, entrepreneurs have a 2.0x to 4.0x cost of capital differential from investors. In essence, given the lack of diversification held by a significant group, the value of these securities would be worth less than if held by those that do not have this concern. This means that the bid and the ask for the security value can be quite dramatic between the two hypothetical groups.

*Figure 1: Optimal Bidding Metrics in Transactions***[[29]](#footnote-30)**



As such, the bidding dynamics in how a transaction would unfold play a huge role with respect to where one might expect a transaction price to close. For early-stage biotechnology companies, such as the Company, it is more reasonable to assume that the market for buying/selling the common shares would be better approximated by the two-bidder representation than the 50-bidder representation. As value is created through program development and specifically positive clinical data, it is our experience that the number of potential interested parties would dramatically increase.

In addition, optionality is not often valued in a transaction. It is only when the nature of the bidding process includes competition from multiple parties that the optionality or real optionality of a potential security’s value gets unlocked. It is unlikely that this is the case for the Company given the very early-stage of its program development.

Combining the perspective that there would not be a strong bidding market for the security and that optionality would not be as strongly considered, the cost of capital associated with the group of sellers would be a more appropriate perspective to take. As such, for the Common Stock, we considered a differential return of approximately 2.5x the expected return by preferred stockholders or investors. The required rate of return for preferred stock reflects approximately 20.0% VC portfolio returns (across the total preferred investment). Such expected VC portfolio returns are generally consistent with observations of historical VC returns. Based on the above-mentioned returns, a non-diversification risk factor of 30.0% was considered in the analysis. Refer to Exhibit H.8 for further details.

**Reconciliation to Latest Preferred Financing**

RNA noted that the combination of assumptions noted above ultimately resulted in an implied Series B price per share of $2.72, 36.5% lower than its original issue price of $4.29, which is reasonable given the shift in the Company’s focus to one program from multiple programs in the Previous Valuation and the failure to raise Series B Tranche II financing. The decrease in the value of the latest preferred financing is in line with the 33.7% decrease in the value of the Common Stock.

**PWERM Conclusion**

Based on the methodology outlined above, we concluded the value of Common Stock to be $0.60/share on a non-marketable, minority basis. Refer to Exhibit B.1 for further details.

Conclusion

The overall equity value is $42.6 million (rounded) as shown in Exhibit A.1, was inferred from the assumptions used in the PWERM Method.

Based on our analysis, it is our opinion that the fair market value and the fair value of the Common Stock of the Company on a non-marketable, minority interest basis as of the Valuation Date is **$0.60 (SIXTY CENTS) per Share**.

*Table 8: Valuation Summary*

|  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- |
| **VALUATION SUMMARY** |  |  | **(USD IN THOUSANDS  EXCEPT PER SHARE PRICE)** | | | |  |
| **Allocation of Value** |  |  | **Common Stock** | **Equity Value** | | **Selected Approach** |  |
| Probability-Weighted Expected Return Method |  |  | $0.60 | $42,640 | | Primary |  |
| **Concluded fair market value of Common  Stock (non-marketable, minority basis)** | | | | | **$0.60** | |  |

The conclusions and opinions expressed in this letter and the accompanying Report are contingent upon the qualifying factors set forth in the Statement of Limiting Conditions attached to the completed Report.

Statement of Limiting Conditions

This Opinion has been prepared pursuant to the following general assumptions and general limiting conditions:

* We assume no responsibility for the legal description of real property or matters including legal or title considerations. For real property included in this appraisal, we were not furnished legal descriptions or other detailed site and improvement drawings. Title to the subject assets, properties, or business interests is assumed to be good and marketable unless otherwise stated.
* The subject assets, properties, or business interests are appraised free and clear of any or all liens or encumbrances unless otherwise stated.
* We assume responsible ownership and competent management with respect to the subject assets, properties, or business interests.
* The information furnished by management is believed to be reliable. However, we issue no warranty or other form of assurance regarding its accuracy.
* We assume that there is full compliance with all applicable Federal, state, and local regulations and laws unless noncompliance is stated, defined, and considered in the appraisal Report.
* We assume that all required licenses, certificates of occupancy, consents, or legislative or administrative authority from any local, state, or national government, private company or organization have been or can be obtained or renewed for any use on which the valuation opinion contained in this Report is based.
* Possession of this valuation Report, or a copy thereof, does not carry with it the right of publication. It may not be used, without our written consent, for any purpose by any person other than the party to whom it is addressed and, in any event, only with proper written qualifications and only in its entirety.
* We, by reason of this valuation, are neither required to give testimony nor to be in attendance in court with reference to the assets, properties, or business interests in question unless arrangements have been previously made.
* This valuation Report has been prepared in conformity with, and is subject to, the requirements of the code of professional ethics and standards of professional conduct of the professional appraisal organizations of which we are members.
* Disclosure of the contents of this valuation Report is governed by the bylaws and regulations of the CFA Institute.
* No part of the contents of this Report, especially any conclusions of value, the identity of the appraisers, or the firm with which the appraisers are associated, shall be disseminated to the public through advertising, public relations, news, sales, or other media without our prior written consent and approval.
* We assume no responsibility for any financial reporting judgments, which are appropriately those of Management. Management accepts the responsibility for any related financial reporting with respect to the assets, properties, or business interests encompassed by this appraisal.

Qualifications

Samuel Renwick, CFA

Sam Renwick provides valuation and advisory services to biopharmaceutical, medical device and equipment, diagnostic companies, and clinical research and manufacturing organizations, as well as other IP-centric technology companies. His experience includes buy-side and sell-side advisory engagements for licensing, financing, and mergers and acquisitions, as well as for tax and financial reporting matters for large public companies to small venture-backed enterprises. Whether developing dynamic, patient flow models for late-clinical therapeutic assets or developing an opinion of value for a security for compliance purposes, Mr. Renwick combines his breadth of industry knowledge with deep expertise in finance and financial models to create compelling communications regarding the value proposition of an asset, portfolio of assets or a company. Mr. Renwick has worked with well over 600 life sciences and technology companies in his career.

Professional Affiliations

* UCLA Anderson Business Honor Society
* CFA Institute
* Chartered Financial Analyst Society of San Francisco
* Member, Fair Value Forum
* Licensing Executive Society

Education

* BA/Economics & Business – Westmont College, Honors
* MBA/Finance – UCLA Anderson, Honors, J. Fred Weston award for Academic Excellence in Finance
* Chartered Financial Analyst (CFA)

Publications

* 409A Administration Handbook Valuation Section – Thomson Reuters, 2014
* Why Your 409A Valuation is Too High – Dis-Incentive Stock Compensation in the Life Sciences – BPM White Paper, May 2013
* Modeling and Forecasting to Communicate the Biotherapeutic Value Proposition – BayBio White Paper, May 2010
* Common Stock Valuation – Tips from the Trade, BayBio NOTES, April 2010
* Defensible Equity Incentive Valuation Opinions Under IRC 409A, Company Newsletter, December 2009
* What is the IRS Doing with IRC 409A, Silicon Valley Bank Newsletter, December 2008
* Eleven of the Top Ten Mistakes to Avoid in Your Options Program, Atlanta CEO Connexions, August 2007

Instruction and Seminars

* Presentation on Valuation and Funding Strategies for Digital Health Companies at the Cedars Sinai Techstars Accelerator, October 2017
* Panelist on Valuation Issues in Early Stage Company Valuations – Fair Value Summit November 2015
* Presentation on Funding Technology Innovation to Caltech Science and Entrepreneurship Group, Pasadena, March 2015
* Presentation to accelerator programs/technology transfer group on Financing Early Stage Technologies – Navigating Valuation Discussions, UC Berkeley, October 2014
* Panelist, Funding Early Stage Ventures, Sand Hill Angels, September 2013
* Panelist on Communicating the Biotech Value Proposition, BayBio Annual Event, South San Francisco, May 2011
* Presentation on the Use of Discount Rates in the PWERM, Fair Value Forum, Palo Alto, November 2008
* Panelist on the Valuation of Biotechnology Companies, Biocom, San Diego, May 2008

Certification

I certify that, to the best of my knowledge and belief:

* The statements of fact contained in this Report are true and correct.
* The reported analyses, opinions, and conclusions are limited only by the reported assumptions and limiting conditions, and are our personal, impartial, and unbiased professional analyses, opinions, and conclusions.
* We have no present or prospective interest in the property that is the subject of this Report, and we have no personal interest with respect to the parties involved.
* We have no bias with respect to the property that is the subject of this Report or to the parties involved with this assignment.
* Our engagement in this assignment was not contingent upon developing or reporting predetermined results.
* Our compensation for completing this assignment is not contingent upon the development or reporting of a predetermined value of direction in value that favors the cause of the client, the amount of the value opinion, the attainment of a stipulated result, or the occurrence of a subsequent event directly related to the intended use of this appraisal.
* Our analyses, opinions and conclusions were developed, and this Report has been prepared, in conformity with the AICPA Statement on Standards for Valuation Services.

Sincerely,



Samuel Renwick, CFA

Exhibits

1. IRS Revenue Ruling 59-60. [↑](#footnote-ref-2)
2. Accounting Standards Codification Topic 718 – Stock Compensation (formerly Statement of Financial Accounting Standards No. 123R, Accounting for Share-Based Payment). [↑](#footnote-ref-3)
3. Source: Information provided by Management. [↑](#footnote-ref-4)
4. Source: Ibid. [↑](#footnote-ref-5)
5. Source: Information provided by Management. [↑](#footnote-ref-6)
6. Source: Information provided by Management. [↑](#footnote-ref-7)
7. Ibid. [↑](#footnote-ref-8)
8. Source: https://www.linkedin.com/in/marshall-fordyce-md-05751846/. [↑](#footnote-ref-9)
9. Source: http://lagunaceoforum.com/wp-content/uploads/2015/10/CDF-Therapeutics.pdf. [↑](#footnote-ref-10)
10. Source: https://www.linkedin.com/in/allen-ebens-4a41113/. [↑](#footnote-ref-11)
11. Source: Information provided by Management. [↑](#footnote-ref-12)
12. Source: <https://www.linkedin.com/in/lauren-pflepsen-frenz-0b2b466/> and information provided by Management. [↑](#footnote-ref-13)
13. Report, “Global Genome Editing Market 2018-2022”, published in April 2018,

    https://www.researchandmarkets.com/reports/4530622/global-genome-editing-market-2018-2022. [↑](#footnote-ref-14)
14. Report, “Genome Editing Market to 2025 – Global Analysis and Forecast by Technology”, published in October 2018,

    https://www.researchandmarkets.com/reports/4669189/genome-editing-market-to-2025-global-analysis#pos-2. [↑](#footnote-ref-15)
15. Report, “Global Genome Editing Market - Segmented by Technology…”, published in March 2018,

    https://www.researchandmarkets.com/reports/4520086/global-genome-editing-market-segmented-by#pos-9. [↑](#footnote-ref-16)
16. Report, “Genome Editing Market to 2025 – Global Analysis and Forecast by Technology”, published in October 2018,

    https://www.researchandmarkets.com/reports/4669189/genome-editing-market-to-2025-global-analysis#pos-2. [↑](#footnote-ref-17)
17. Report, “Global Genome Editing Market - Segmented by Technology…”, published in March 2018,

    https://www.researchandmarkets.com/reports/4520086/global-genome-editing-market-segmented-by#pos-9. [↑](#footnote-ref-18)
18. Press Release, “CRISPR Cas9 Genome editing market worth $5.3 billion by 2025”, published on November 23, 2018, https://www.aheadintel.com/crispr-cas9-genome-editing-market/. [↑](#footnote-ref-19)
19. Report, “Hemoglobinopathies Market Insights | Emerging Trends & Demand | Forecast To 2022” published in October 30, 2018, https://marketreportr.wordpress.com/2018/10/30/hemoglobinopathies-market-insights-emerging-trends-demand-forecast-to-2022/. [↑](#footnote-ref-20)
20. Report, “Hemoglobinopathies Market Insights | Emerging Trends & Demand | Forecast To 2022” published in October 30, 2018, https://marketreportr.wordpress.com/2018/10/30/hemoglobinopathies-market-insights-emerging-trends-demand-forecast-to-2022/. [↑](#footnote-ref-21)
21. Report, “Sickle Cell Disease Treatment Market (2018-2023) Size…” published in September 24, 2018, https://www.businesswire.com/news/home/20180924005640/en/Sickle-Cell-Disease-Treatment-Market-2018-2023-Size. [↑](#footnote-ref-22)
22. Report, “Global Sickle Cell Disease Treatment Market 2019-2023”, published in January 2019, https://www.researchandmarkets.com/reports/4747488/global-sickle-cell-disease-treatment-market-2019#pos-2. [↑](#footnote-ref-23)
23. Report, “Sickle Cell Disease Treatment Market (2018-2023) Size…” published in September 24, 2018, https://www.businesswire.com/news/home/20180924005640/en/Sickle-Cell-Disease-Treatment-Market-2018-2023-Size. [↑](#footnote-ref-24)
24. Press Release, “The FDA Encourages New Treatments for Sickle Cell Disease”, Updated on June 18, 2018 https://www.fda.gov/ForConsumers/ConsumerUpdates/ucm418232.htm. [↑](#footnote-ref-25)
25. Source: Information provided by Management. [↑](#footnote-ref-26)
26. Originally created in 1973, the Black‐Scholes option pricing model attempts to calculate the price of an option by considering several key factors, such as the underlying security price, exercise price, expiration date, risk‐free rate and the standard deviation of a stock’s return. Numerous assumptions underlie Black‐Scholes, including but not limited to, the log‐normal distribution of returns, and static risk‐free rates and volatility. [↑](#footnote-ref-27)
27. The binomial model was first proposed by Cox, Ross and Rubinstein in 1979, and essentially uses a “discrete-time” (lattice based) model of the varying price over time of the underlying financial instrument. In general, such models do not have closed-form solutions. [↑](#footnote-ref-28)
28. Gary R. Trugman, Understanding Business Valuation, (American Institute of Certified Public Accounts, 2002), pg. 325. [↑](#footnote-ref-29)
29. Source: Article by Harvard Business Review from December 2009 Issue, https://hbr.org/2009/12/negotiation-auction-a-deal-makers-guide. [↑](#footnote-ref-30)