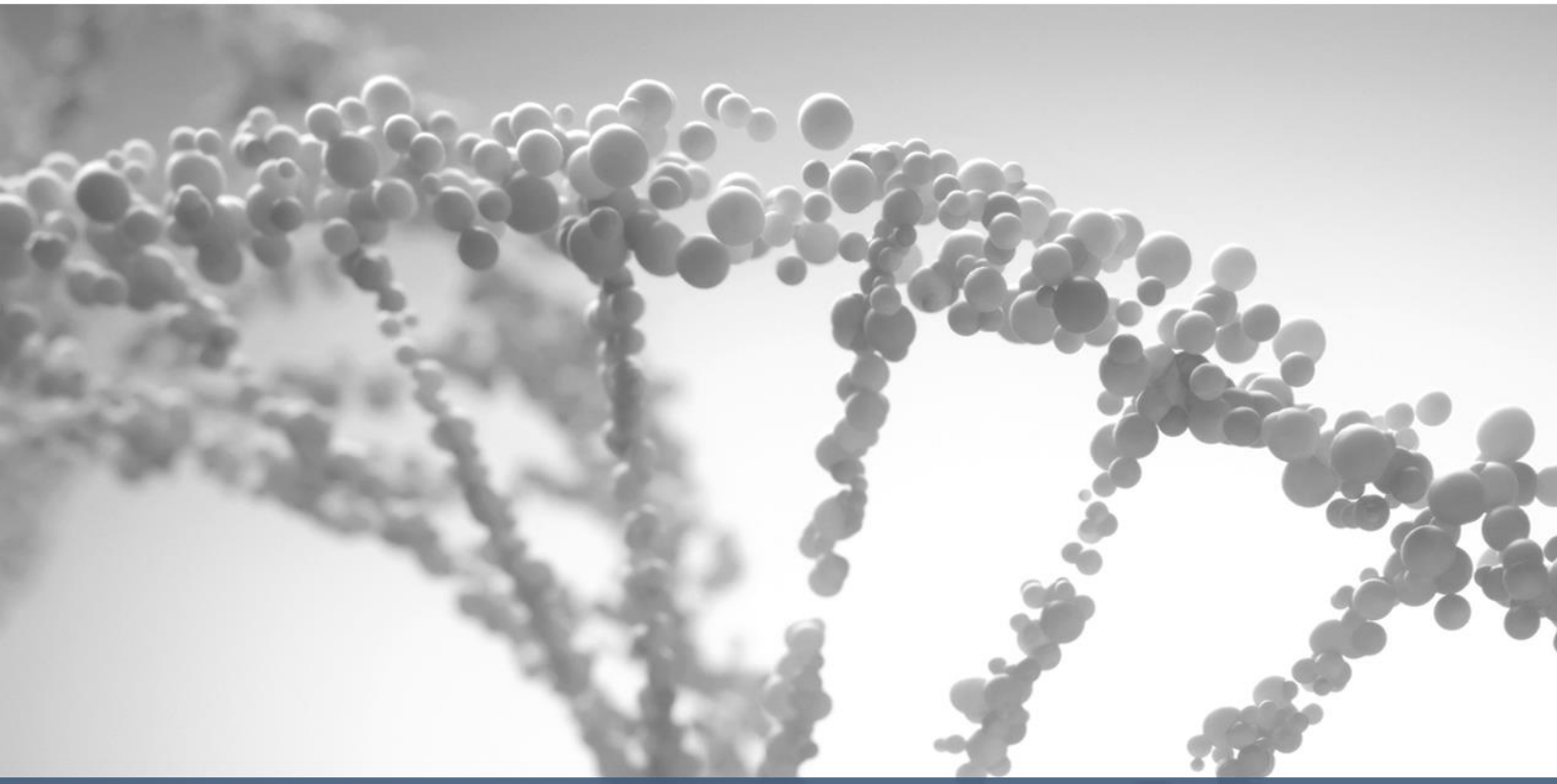
FORECASTING | MODELING | VALUATION | MARKET ASSESSMENT



RaNA Therapeutics, Inc.'s Acquisition of the MRT Business Unit of Shire Human Genetic Therapies, Inc.

PURCHASE PRICE ALLOCATION AS OF DECEMBER 22, 2016

Report Date: July 26, 2017

RANA THERAPEUTICS

RNA Capital Advisors
Built for the art and science of the deal

July 26, 2017

Mr. Robert Prentiss
Director of Finance & Accounting

RaNA Therapeutics, Inc.
200 Sidney Street
Suite 310
Cambridge, MA 02139

Dear Mr. Prentiss:

In response to the engagement letter, RNA Advisors, LLC dba RNA Capital Advisors ("RNA" or "we") has completed an analysis to determine the fair value of certain intangible assets acquired and liabilities assumed as part of the acquisition of the messenger RNA business unit ("Shire MRT" or the "Transferred Assets" or the "Subject Interest") from Shire Human Genetic Therapies, Inc. ("Shire" or the "Seller") by RaNA Therapeutics, Inc. ("RaNA Therapeutics", the "Company", or the "Buyer") as of December 22, 2016 (the "Valuation Date").

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") for the allocation of purchase price for financial reporting purposes. This analysis has been performed in recognition of Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 805 – Business Combinations ("ASC 805") (formerly known as Statement of Financial Accounting Standards No. 141R, Business Combinations ("SFAS 141R")) and FASB ASC 820 - Fair Value Measurements and Disclosures ("ASC 820") (formerly Statement of Financial Accounting Standards No. 157, Fair Value Measurements ("SFAS 151")). 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 valuation engagement was conducted in accordance with the Statement on Standards for Valuation Services No.1 ("SSVS 1") - "Valuation of a Business, Business Ownership Interest, Security, or Intangible Asset" of the American Institute of Certified Public Accountants ("AICPA")".[1]

[1] This AICPA Practice Aid, which was initially developed in 2001, was revised in 2014 by the AICPA staff and IPR&D Task Force. Source: https://www.kpmg.com/CN/en/IssuesAndInsights/ArticlesPublications/Newsletters/Defining-Issues/Documents/Defining-Issues-O-1401-04.pdf.

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 value of the Subject Interest as of the Valuation Date is as follows:

Table 1: Valuation Summary

VALUATION SUMMARY	(USD IN THOUSANDS)
Asset	**Value Indication**
Tangible Assets	2,416.2
Intangible Assets	
IPR&D Assets	
CF Program	42,291.4
OTC Program	18,559.0
MRT Platform Programs	45,991.9
Lease Agreement	65.5
Goodwill	
Assembled Workforce	686.7
Goodwill	4,649.2
Concluded Fair Value of the Subject Interest	**114,660.0**
Purchase Consideration	**Value Indication**
Total Value of Shares Issued to Shire	49,539.8
Payment to MTS	2,500.0
Contingent Consideration	
Royalty and Milestone Payments	62,665.8
Anti-Dilution Rights	(42.9)
Total Contingent Consideration	**62,662.9**
Total Purchase Consideration	**114,660.0**

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

DRAFT

Sam Renwick, CFA
Primary Valuation Analyst

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Engagement Overview

PURPOSE

RNA has completed an analysis of the Company and the Subject Interest as of the Valuation Date to determine the fair value of the Subject Interest. This analysis has been performed in recognition of the SSVS1 issued by the AICPA. This analysis uses the methods and techniques outlined in ASC 805 and ASC 820, which are relevant to the valuation of the Subject Interest.

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:

(i) Discussed with Management the history and nature of the Subject Interest, as well as the specific industry conditions affecting the Subject Interest and the underlying business, including but not limited to the Subject Interest's prospective earnings capacity;
(ii) Reviewed the Common Stock Valuation Opinion (defined below);
(iii) Reviewed the Asset Purchase Agreement (defined below) and related agreements for the Transaction (defined below);
(iv) Reviewed the Lease Agreement (defined below) and survey of comparable leases as provided by Management;
(v) Reviewed the MIT Agreement (defined below);
(vi) Reviewed the summary of other third-party agreements related to the MRT Platform (defined below);
(vii) Discussed the costs associated with Assembled Workforce (defined below);
(viii) Reviewed Amendment #2 to the Engagement Letter and Assumption Agreement signed between Shire, MTS (defined below), and the Company ("Amendment #2");
(ix) Reviewed the fixed assets listing for the Subject Interest as prepared by Management;
(x) Reviewed a capitalization summary of the Company as prepared by Management;
(xi) Reviewed a copy of the Company's Articles of Incorporation;
(xii) Reviewed copies of certain documents pertaining to various securities underlying the Company's capital structure, such as preferred and common stock;
(xiii) Reviewed certain publicly available financial data for companies that we deemed comparable to the Company and Shire MRT;
(xiv) Conducted research concerning the economic conditions and outlook for the US economy generally as of the Valuation Date; and
(xv) Conducted other studies, analyses and inquiries, as we deemed appropriate.

The assets and liabilities considered as a part of the analysis have been discussed in detail in the "Assets/Liabilities Overview" Section below.

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 "CF", as used herein, refers to cystic fibrosis.

The term "cGMP" refers to the Current Good Manufacturing Practice regulations enforced by the FDA (defined below).

The term "Common Stock", as used herein, refers to the Company's common stock.

The term "Common Stock Valuation Opinion", as used herein, refers to the valuation of the Company's Common Stock performed by RNA as of the Valuation Date.

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

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

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

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 "IPR&D", as used herein, refers to in-process R&D (defined below),

The term "IPR&D Assets", as used herein with respect to this Opinion, refers to the part of the Transferred Assets, which includes two programs focused on CF and OTC (defined below) and the MRT Platform (individually an "IPR&D Asset", collectively the "IPR&D Assets").

The term "Lease Agreement", as used herein, refers to the lease agreement signed between Shire and 128 Spring Street Lexington, LLC as of April 16, 2014.

The term "lncRNA", as used herein, refers to long non-coding RNA.

The term "LNP", as used herein, refers to liquid natural polymer.

The term "LSD", as used herein, refers to lysergic acid diethylamide.

The term "MIT Agreement", as used herein, refers to the exclusive patent license agreement signed between Shire and the Massachusetts Institute of Technology ("MIT") effective as of November 1, 2013,

related to the licensing out of patent rights by MIT, related to "Amino Acid, Peptide, Polypeptide-Lipids ("APPL") Derivatives and Uses Thereof", to Shire.

The term "mRNA", as used herein, refers to messenger RNA (as defined below).

The term "MRT", as used herein, refers to mRNA replacement therapy.

The term "MRT Platform", as used herein, refers to Shire MRT's proprietary MRT platform.

The term "NASH", as used herein, refers to non-alcoholic steatohepatitis.

The term "NPV", as used herein, refers to net present value.

The term "OTC", as used herein, refers to ornithine transcarbamylase.

The term "PKU", as used herein, refers to phenylketonuria.

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, "RNAi", as used herein, refers to RNA interference.

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

The term "siRNA", as used herein, refers to short interfering RNA.

The term "UCD", as used herein, refers to urea cycle disorders.

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.

The term "USPTO", as used herein, refers to the United States Patent and Trademark Office.

The term "UTR", as used herein, refers to untranslated region.

The term "WW", as used herein, refers to worldwide.

Standard of Value

The intangible assets and liabilities included in our analysis were valued incorporating the concept of highest and best use in accordance with ASC 805, which uses the Fair Value definition in ASC 820, *Fair Value Measurement*, from the viewpoint of a market participant.

ASC 805 – BUSINESS COMBINATIONS

ASC 805 defines Business Combinations as follows:

"A transaction or other event in which an acquirer obtains control of one or more businesses. Transactions sometimes referred to as true mergers or merger of equals also are business combinations."

ASC 805-20-30-1 provides that the *"acquirer shall measure the identifiable assets acquired, the liabilities assumed, and any non-controlling interest in the acquiree at their acquisition-date fair values."*

ASC 805-30-30-1 indicates that *"goodwill should be recorded as the sum of the (a) consideration transferred, (b) fair value of any non-controlling interest, and (c) fair value of the acquirer's previously held interest in the acquiree, if any, less the acquisition-date fair value of the net assets acquired."*

DEFINITION OF FAIR VALUE

For financial reporting purposes, the appropriate standard of value is fair value, which is defined in ASC 820 (formerly Statement of Financial Accounting Standards No. 157 Fair Value Measurements) as:

"The price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market transactions at the measurement date" ("Fair Value")."

ASC 820 states that a fair value measurement assumes the highest and best use of the asset by market participants, considering the use of the asset that is physically possible, legally permissible, and financially feasible at the measurement date. In broad terms, highest and best use refers to the use of an asset by market participants that would maximize the value of the asset or the group of assets within which the asset would be used. Moreover, the highest and best use is based on the use of the asset by market participants, even if the intended use of the asset by the reporting entity is different.

The highest and best use of the asset by market participants establishes the valuation premise used to measure the fair value of the asset: (a) in-use, if the asset would provide maximum value to market participants principally through its use in combination with other assets as a group, installed or otherwise configured for use; or (b) in exchange, if the asset would provide maximum value to market participants principally on a standalone basis.

Company Overview

BACKGROUND

RaNA Therapeutics, headquartered in Cambridge, Massachusetts, was founded in 2011 by Arthur M. Krieg, Jeannie T. Lee[2] and Jean-François Formela[3]. RaNA Therapeutics pioneers in the discovery of a new class of RNA-targeted medicines that selectively activate protein expression within the body's own cells. The Company's drugs work epigenetically to precisely upregulate the expression of beneficial proteins in order to treat or prevent disease.[4]

Shire MRT, headquartered in Lexington, Massachusetts, was founded in 2008. Shire MRT had been funded by Shire since its incorporation and had 15 employees as of the Valuation Date. The lead indications of Shire MRT target CF, as well as OTC deficiency (a type of UCD). Shire MRT has developed the MRT Platform that has the potential to address unmet disease targets. Shire MRT's Proprietary cGMP manufacturing produced the highest quality mRNA at largest scale available in the industry, and had produced a single proof-of-concept ("POC") batch of 100 grams.[5]

DEAL OVERVIEW[6]

On December 22, 2016, the Company acquired the MRT business unit of Shire in an asset purchase transaction in accordance with Section 351 of the Internal Revenue Code (the "Transaction"). The following was determined based on the asset purchase agreement signed by and between the Company and Shire on December 22, 2016 ("Asset Purchase Agreement").

As part of the total consideration for the deal, Shire would receive Common Stock, as well as other forms of contingent consideration ("Contingent Payments").

As part of the Transaction, the Company was required to raise $100.0 million in equity financing in one or multiple tranches (each, a "Tranche", and collectively the "Tranches") so that the Company could continue developing the programs and technology underlying the Transferred Assets. The first Tranche of Series C financing ("Series C – Tranche I" or "Tranche I") totaling $51.0 million, was closed as on the Valuation Date, and based on discussions with Management there was a very high probability of the second Tranche of $49.0 million ("Series C – Tranche II" or "Tranche II") closing.

As per the terms of the Asset Purchase Agreement, if the value of Tranche I was less than $100.0 million, the Company would be required to use $50.0 million of the net proceeds of Tranche I solely for the

[2] Source: https://relationshipscience.com/rana-therapeutics-inc-0249659.
[3] Source: Company website, http://ranarx.com/about-rana/.
[4] Source: Information provided by Management
[5] Source: Information provided by Management.
[6] Source: Asset Purchase Agreement and information provided by Management.

activities related to the Transferred Assets. Furthermore, on the raise of Tranche II, the number of newly issued shares of Common Stock should be such that Shire owns either:

(i) 18.0% of the Common Stock calculated on an as-converted and fully diluted based or;
(ii) If less, Common Stock representing 19.9% of the voting power of all outstanding Common Stock (excluding unvested restricted stock).

Based on the Asset Purchase Agreement, 32,308,347 shares of Common Stock ("Shire – Tranche I") were issued as purchase consideration for the Transaction as of the Valuation Date. Based on the terms detailed above, RNA estimated 6,644,384 shares of Common Stock ("Shire – Tranche II") as the newly issued shares of Common Stock. We calculated the additional shares based on 18.0% of common stock on an as-converted and fully diluted basis, as well as based on the 19.9% of voting stock. However, we considered the additional shares calculated based on the former in our analysis, since the voting stock calculation changes as restricted shares vest. Furthermore, we noted that both calculations result in fairly similar share counts that do not have a material impact on this valuation. Refer to the Common Stock Valuation Opinion for further details.

Based on the terms of the Asset Purchase Agreement, the Company is required to make the following Contingent Payments:

(i) A one-time payment of $35.0 million upon the first commercial sale of any CF transmembrane conductance regulator ("CFTR") mRNA therapeutics program in the US or the European Union ("EU"), including the United Kingdom ("UK");
(ii) A one-time payment of $10.0 million upon the first commercial sale of any non-CFTR MRT product in the US or the EU; (collectively (i) and (ii), the "Approval Milestone Payments"); and
(iii) A one-time payment of $25.0 million upon the first achievement of the aggregate annual net revenues of any MRT product equaling or being greater than $500.0 million (the "Sales Milestone" and the related payment - "Sales Milestone Payment").

In addition to the above one-time payments, the Company is also required to make the following royalty payments (the "Royalty Payments"):

(i) Quarterly payments equal to 4.0% of net sales ("Royalty Payment Rate") of each MRT product in each country during the Royalty Earn-Out Period (defined below), which is the period beginning from the first commercial sale and lasting until the later of:
    a. Expiration of the valid claim for the MRT product ("Patent"); and
    b. 10 years from the first commercial sale (or "Royalty Earn-Out Period").
(ii) The Royalty Payment Rate will be reduced by 15.0%, if the Patent for the MRT product expires in a country for which the Royalty Earn-Out Payments are being considered.

In addition to the above payments, the Company will also make the following payments to MTS Securities, LLC ("MTS"), the investment banker for the Transaction, pursuant to the Amendment #2 effective as of the Valuation Date:

(i) $2.0 million in cash ("Cash Fee") payable on the Valuation Date; and
(ii) Common Stock equal to the quotient obtained by dividing $500,000 by price per share equal to the fair value of the Common Stock to be determined following the Valuation Date.

Based on the fair value of the Common Stock concluded in Common Stock Valuation Opinion, RNA estimated that the Company would be required to issue 393,146 shares of the Common Stock to MTS. Refer to the Common Stock Valuation Opinion for further details.

ACQUIRED PRODUCT PIPELINE / PRODUCTS AND TECHNOLOGY

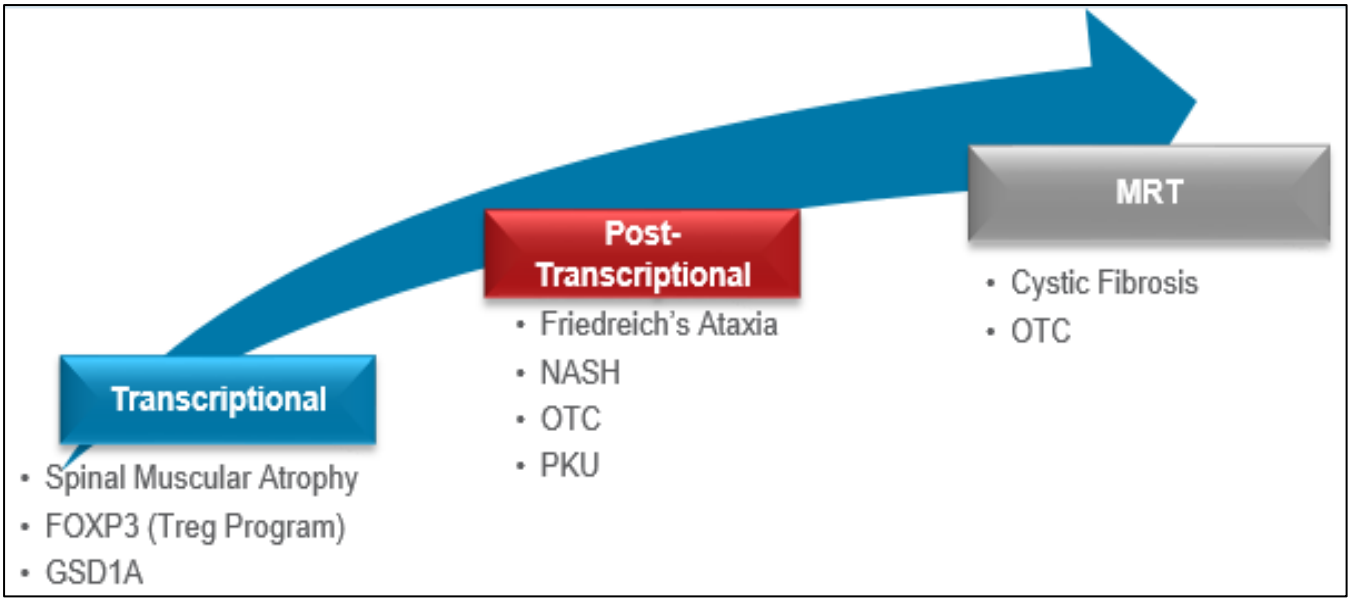
Shire MRT's proprietary MRT Platform will help demonstrate mRNA delivery via intrathecal, intra-ocular and intra-articular administration. Generally, manufacturing scalability and drug delivery are the most common hindrances faced by the developers of MRT, however, the MRT Platform has overcome both these challenges. The MRT Platform provides high quality mRNA with a large scale of production. Furthermore, access to most potent drug delivery agents via licensing and agreements with the academic research institution will help strengthen the drug delivery technology.

The MRT Platform has established POC efficacy in a wide range of animal models such as mouse, rat, rabbit, and pig, and has also demonstrated efficacy in multiple disease models and established safe and effective dosing using unmodified mRNA in small and large animals. The MRT Platform has the potential for broad therapeutic applicability, as mRNA produces the desired protein endogenously where organs act as protein production factories for local or systemic therapy. The applications of the MRT Platform include chronic therapeutics, immunotherapy, and gene correction.[7]

The MRT Platform technology has the potential to apply to a broad range of diseases; however, its initial focus is on developing treatments for CF and OTC. These indications meet important criteria for the first MRT development programs. The prioritized disease areas possess high unmet need, strong scientific rationale, and the potential for rapid clinical de-risking of the MRT approach. To accelerate the drug development process in these two areas, the Company closed the Transaction.

[7] Source: Ibid.

Figure 1: Summary of RNA platforms

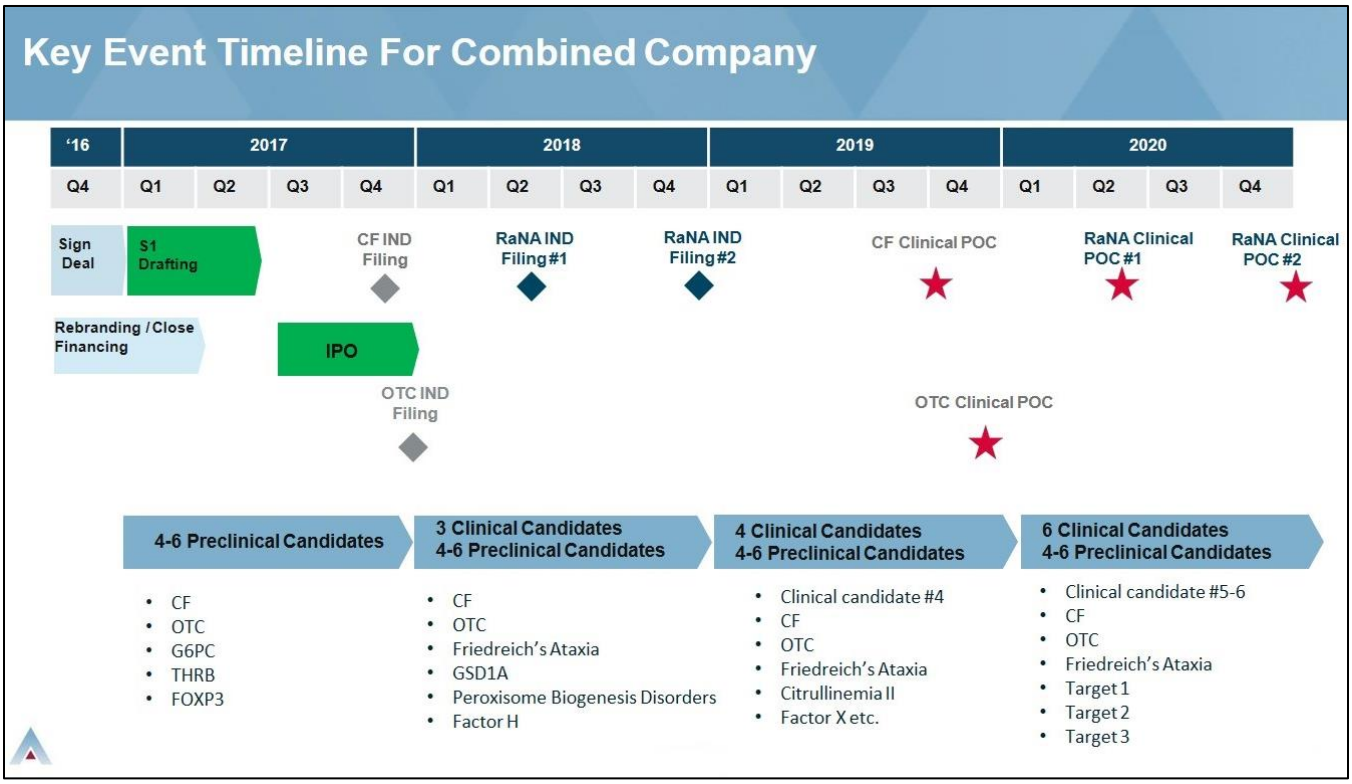


The Company expects that the Transaction would enable it to expand its therapeutic approach and will provide it access to a wider combined drug program portfolio. The combined entity would contain three distinct RNA platforms – transcriptional, post-transcriptional, and MRT. All the platforms are protected by extensive IP portfolios.

The Company expects to file an IND for its CF drug by the fourth quarter of 2017, and the IND filing for OTC is expected by the first quarter of 2018, as noted in the figure below:[8]

[8] Source: Information provided by Management.

Figure 2: Key Events Timeline



INTELLECTUAL PROPERTY

The MRT Platform has a broad IP portfolio, addressing all areas of mRNA development, with more than 35 patent families and over 50 patent applications (245 filings worldwide). These patents are related to the following technologies:

(i) Production of proteins, antibodies, nucleases, virus;
(ii) Delivery to liver, pulmonary, central nervous system, ocular, articular;
(iii) Treatment of UCDs, LSDs, CFTR, Pompe disease, PKU;
(iv) Composition of matter for mRNA / lipids / polymers / LNP;
(v) Manufacturing processes for mRNA / LNP / polymers / lipids; and
(vi) Sequences, new constructs, chemistries.

Several of the patents granted include those for sequences (UTRs, CFTR), CF treatment, Fabry disease.[9]

[9] Source: Information provided by Management.

MANAGEMENT TEAM[10]

The key members of the Management team of the Company are:

Jeannie T. Lee, M.D., PhD – *Co-Founder*
Dr. Lee is the Scientific Co-Founder of the Company. She is currently an Investigator of the Howard Hughes Medical Institute and Professor of Genetics (and Pathology) at Harvard Medical School and the Massachusetts General Hospital ("MGH"). Dr. Lee specializes in the study of epigenetic regulation by lncRNA. Using X-chromosome inactivation ("XCI") as a model, her laboratory has made several contributions to our understanding of RNA-directed chromatin change, including the role of RNA in targeting polycomb complexes and the mechanisms of repression by antisense RNA. She received her A.B. in Biochemistry and Molecular Biology from Harvard University, where she worked on antisense repression of transposition with Dr. Nancy Kleckner. She obtained M.D.-PhD degrees from the University of Pennsylvania School of Medicine, where she studied epigenetic regulation of X-linked diseases with Dr. Robert L. Nussbaum. At the Whitehead Institute/Massachusetts Institute of Technology, her postdoctoral work with Dr. Rudolf Jaenisch delineated the X-inactivation center. She has served as the Chief Resident of Clinical Pathology at MGH, and received both the Basil O'Connor Scholar Award (March of Dimes) and the Pew Scholars Award (Pew Foundation) as a young investigator. Dr. Lee has been awarded a Distinguished Graduate Award of the University of Pennsylvania School of Medicine, and currently holds a MERIT Award from the National Institutes of Health ("NIH"). She is also the recipient of the 2010 Molecular Biology Prize from the National Academy of Sciences, US, is a Fellow of the American Association for the Advancement of Science ("AAAS"), and serves on the Board of Directors for the Genetics Society of America.

Jean-François Formela, M.D. – *Co-Founder*
Dr. Formela is a Partner and Co-Founder at the Company, and focuses on new advances in biology and drug discovery technologies as well as novel therapeutics. He joined the Company's Board of Directors in 2011, and also serves on the boards of Ataxion, F-star, Intellia Therapeutics and Navitor Pharmaceuticals. He is the chairman of Egalet and Spero Therapeutics. Dr. Formela has been involved in the formation of companies such as Adnexus (acquired by Bristol Myers-Squibb), ArQule, Annovation Biopharma (acquired by The Medicines Company), Arteaus Therapeutics (acquired by Lilly), Cellzome (acquired by GlaxoSmithKline), deCODE (acquired by Amgen), Exelixis, MorphoSys, NxStage, SGX Pharmaceuticals (acquired by Lilly), and ZappRx. He was also an investor in Achillion, CoStim Pharmaceuticals (acquired by Novartis) and Horizon Pharma as well as a board member of Biochem Pharma (acquired by Shire) and Novexel (acquired by AstraZeneca).

Dr. Formela joined Atlas Venture in 1993 to build the US life sciences franchise. Prior to joining Atlas, Dr. Formela worked at Schering-Plough, where he directed US Phase IV studies in all therapeutic areas. Before that, he was responsible for the marketing of Intron A, Schering-Plough's alpha-interferon. Dr. Formela began his career as a medical doctor and practiced emergency medicine at Necker University Hospital in Paris.

[10] Source: Company's website, http://ranarx.com/about-rana/.

Dr. Formela is a member of the Massachusetts General Hospital Research Advisory Council and is a former trustee of the Boston Institute of Contemporary Art. He received his M.D. from Paris University School of Medicine, and his MBA from Columbia University.

Arthur M. Krieg, M.D. – *Co-Founder*

Dr. Krieg co-founded the Company in 2011 and served as the Chief Executive Officer ("CEO") until January 2014, when he transitioned into a Senior Advisor role. He brings more than 20 years of experience in oligonucleotide R&D in academia, biotech, and pharma environments. Dr. Krieg was the Chief Scientific Officer ("CSO") of Pfizer's Oligonucleotide Therapeutics Unit from 2008 to 2011, and was formerly CSO, Executive Vice President ("VP") R&D, and Co-Founder of Coley Pharmaceutical Group, prior to its acquisition by Pfizer in 2008. Dr. Krieg discovered the immune stimulatory CpG DNA motif in 1994, which led to a new approach to immunotherapy and vaccine adjuvants. Based on this technology, he co-founded Coley Pharmaceutical Group in 1997, discovering and taking four novel oligonucleotides into clinical development, the most advanced of which is currently in Phase III trials (at GlaxoSmithKline). Dr. Krieg was a Co-Founder of the first antisense journal, Oligonucleotides, which he edited for 16 years, and he co-founded the Oligonucleotide Therapeutic Society. He is a Director for Cytos Biotechnology and a member of the Scientific Advisory Board for Mirna Therapeutics.

Dr. Krieg received his M.D. from Washington University, completed a residency in Internal Medicine at the University of Minnesota and a Rheumatology Fellowship at the NIH, and then joined the University of Iowa, becoming Professor of Internal Medicine in the Division of Rheumatology. He has published more than 240 scientific papers and is co-inventor on 44 issued US patents covering oligonucleotide technologies.

Ronald C. Renaud, Jr. – *CEO*

Mr. Renaud has been the Company's CEO since 2014. Formerly, Mr. Renaud was with Idenix Pharmaceuticals from 2007 to 2014, where he served as the Chief Financial Officer ("CFO"), Chief Business Officer, President and CEO. Under his leadership, this company refocused its drug discovery and development efforts on nucleotide prodrugs to treat hepatitis C virus, streamlined operations to better enable cross-functional collaboration and employee engagement, which culminated in its acquisition by Merck for $3.85 billion in August 2014. Prior to joining Idenix Pharmaceuticals, Mr. Renaud served as Senior VP and CFO of Keryx Biopharmaceuticals. Before joining Keryx Biopharmaceuticals, Mr. Renaud was a biotechnology equity research analyst at J.P. Morgan, Schwab Soundview and Bear Stearns. He also spent more than five years at Amgen, where he held positions in clinical research, investor relations and finance.

Mr. Renaud holds a B.A. from St. Anselm College and an MBA from the Marshall School of Business at the University of Southern California. In addition to serving on RaNA Therapeutics's Board of Directors, Mr. Renaud is currently also a board member of PTC Therapeutics and Akebia Therapeutics.

Thomas McCauley, PhD – *CSO*

Dr. McCauley has been the Company's CSO since 2016. Formerly, Dr. McCauley served as VP and Head of Global Nonclinical Development at Shire Pharmaceuticals. In this role, Dr. McCauley was responsible for leadership of the Global Nonclinical and Bioanalytical Development departments, including Pharmacology, Metabolism and Pharmacokinetics, Toxicology, and Clinical/Nonclinical Bioanalytical and Biomarker Development groups. Prior to joining Shire, Dr. McCauley was Head of Nonclinical and Clinical Pharmacology and Pharmacokinetics at Inotek Pharmaceuticals Corp. and was a founding scientist and group leader for Nonclinical and Clinical Drug Metabolism and Pharmacokinetics at Archemix Corp. In addition to his current focus on developing novel therapeutics for rare diseases, Dr. McCauley has over 16 years of experience in the biotechnology and pharmaceutical industries in preclinical and clinical discovery and development of biologic and small molecule drugs for indications in cardiovascular disease, hematology, oncology, inflammation and ophthalmology. He has authored more than 40 scientific publications, book chapters and patents.

Dr. McCauley holds undergraduate and graduate degrees from Cornell University, and received his PhD. from the University of Alabama at Birmingham.

Balkrishen Bhat, PhD – *VP, Chemistry*

Dr. Bhat has been the Company's VP of Chemistry since 2015. Dr. Bhat has more than 25 years of experience in the discovery and development of nucleosides, oligonucleotides for antisense, RNAi and microRNA platform technologies. He joined the Company from Regulus Therapeutics, where he was Senior Director and Department Head of Chemistry for more than six years. In this role, he built world-class oligonucleotide synthesis capability and led the efforts in compound identification and optimization of microRNA modulators for therapeutics applications. Dr. Bhat played a key role in the discovery and development of RG101 for the treatment of hepatitis C, RG012 for the treatment of Alport Syndrome and RG125 to treat NASH. Dr. Bhat also led the efforts to develop new chemical entities and cell based delivery systems to various cell and tissue types. Prior to Regulus, Dr. Bhat spent 13 years at Isis Pharmaceuticals where he held various positions including Director of Medicinal Chemistry. He played a key role in creating new chemical entities in the field of nucleosides, nucleotides and backbone modifications including bicyclic nucleic acids. Dr. Bhat has co-authored over 90 publications and is co-inventor of more than 100 patent applications (50 issued US patents).

Dr. Bhat received his PhD in Medicinal Chemistry from Central Drug Research Institute, Lucknow, India and held his postdoctoral fellowship at University of Illinois Urbana-Champaign.

Brian Fenton – *VP, Corporate Development*

Mr. Fenton has been the Company's VP of Corporate Development since 2015. With more than 25 years of experience in the biotechnology industry with broad transaction experience in business development and alliance management, Mr. Fenton joined the Company from Shire where he most recently served as the Head of Business Development, Neuroscience, supporting the Commercial Business Unit. In this role, he was also responsible for managing Shire's portfolio investments through its former Strategic Investments Group. Mr. Fenton also spent several years in Shire's Rare Diseases Business Development

group where he led and executed several strategic transactions for the company. Prior to Shire, Mr. Fenton spent over 11 years in various roles in Business Development and Alliance Management at Idenix Pharmaceuticals, Codexis and Abbott Laboratories. He has been a co-chair for over 20 years with MassBio serving on several committees and most recently served on the organization's Forum Advisory Board. Mr. Fenton has an undergraduate degree in Biochemistry from the University of Massachusetts/Amherst, an M.S., in chemical engineering from the University of Virginia and an MBA from the Worcester Polytechnic Institute.

Nelson Chau, PhD – *VP, Drug Discovery*

Dr. Chau joined the Company in 2014 and has been serving as VP of Drug Discovery since 2016. He is responsible for all facets of early drug discovery efforts at the Company. Formerly, Dr. Chau was a Research Director at Regulus Therapeutics Inc., a biopharmaceutical company leading the discovery and development of microRNA-based therapeutics. His group was responsible for identifying and validating therapeutic and biomarker microRNA targets and supporting the advancement of multiple therapeutic programs in oncology, fibrosis and metabolic diseases. Such efforts contributed to the advancement of multiple development candidates into the clinic (RG-101, RG-012 and RG-125). Prior to joining Regulus Therapeutics, Dr. Chau was a Senior Scientist at Rosetta Inpharmatics, a subsidiary of Merck Research Laboratory and studied signal transduction pathways in cancer cells using the system biology approach including siRNA functional genomic and global gene expression profiling approaches.

Dr. Chau obtained his PhD at the Johns Hopkins University School of Medicine focused on understanding the molecular mechanism of apoptosis. He was a Damon Runyon Post-Doctoral Fellow at the University of California, San Diego where he utilized microarray profiling to investigate the regulation of gene expression by a tumor suppressor protein, retinoblastoma protein. Dr. Chau is an author of 35 peer-reviewed publications and an inventor of 5 patents and patent applications.

Paula Cloghessy – *VP, Human Resources*

Ms. Cloghessy has been the Company's VP of Human Resources ("HR") since July 2016 and is responsible for HR and organizational development. With 20 years of experience as a senior HR leader, Ms. Cloghessy brings broad-based business experience in biotech and pharmaceutical industries to her role at the Company, including specific expertise in developing HR programs and systems that support aggressive organizational strategy, growth, profitability objectives and innovation. Ms. Cloghessy joined the Company from Joule Unlimited Technologies, Inc. where she most recently served as VP, HR. In this role, she was responsible for leading the company's people strategy and talent architecture in a multi-site global organization. Ms. Cloghessy led the full employee life cycle including a focus on talent acquisition and development, total rewards, organizational development, and employee relations. Prior to Joule Unlimited Technology, Inc., she previously held senior HR leadership roles at Interleukin Genetics, NUCRYST Pharmaceuticals and the Immune Disease Institute (formerly CBR Institute for Biomedical Research), a Harvard Medical School affiliate.

Ms. Cloghessy holds a B.A. in Psychology from the University of Massachusetts and holds the professional Senior Professional in Human Resources and Society for Human Resource Management – Senior Certified Professional designations in the field of HR.

Paul Burgess – *General Counsel*
Mr. Burgess began serving as the Company's General Counsel in 2016. He brings over 20 years of experience in the biotech and pharmaceutical industry with expertise in corporate law, licensing and intellectual property. Prior to joining the Company, Mr. Burgess held senior legal roles at Scholar Rock, Civitas Therapeutics and BIND Therapeutics. He also worked as a lawyer at Johnson & Johnson, Logical Therapeutics and Transform Pharmaceuticals. Prior to becoming a lawyer, Mr. Burgess worked as a scientist at Genetics Institute.

He received his Juris Doctor from Northeastern University, M.S. in Pharmacology from Northeastern University, and B.S. in Biology from Merrimack College.

ASSETS/LIABILITIES OVERVIEW

The following assets were identified in the Transaction, and will be transferred to the Buyer.

Tangible Assets

(i) Current Assets (including inventory): We have not valued inventory separately since Management indicated that the book value of inventory has been estimated to be zero; and

(ii) Long Term Tangible Assets: Includes fixed assets and tenant improvements related to the Lease Agreement as discussed below. We considered the book value of fixed assets as of the Valuation Date. For the tenant improvements, we calculated the book value to be assigned based on the remaining useful life of the tenant improvement assets as a proportion of the remaining life of the lease since the Company will not have access to the tenant improvements once the lease expires. Refer to Exhibit E.3 for details on the tenant improvement assets.

Intangible Assets

The intangible assets can be further classified as follows:

IPR&D ASSETS

The IPR&D Assets include two programs focused on CF (the "CF Program") and OTC deficiency (the "OTC Program"), which were in the preclinical stage as of the Valuation Date. In addition, the IPR&D Assets also include the MRT Platform, and the Company plans to develop more programs using the MRT Platform. For purposes of determining the fair value of the MRT Platform, we have assumed three other programs (one in the discovery stage as of the Valuation Date, and the other two not yet identified but expected to be in the future given the nature of platform technologies), which would be developed through the MRT Platform (individually "Pipeline Program #1", "Pipeline Program #2", "Pipeline Program #3", and collectively the "MRT Platform Programs"). Furthermore, for the OTC Program, we adjusted payments related to the MIT Agreement as mentioned in the "MIT Agreement" section below.

LEASE AGREEMENT

The Lease Agreement was executed on April 16, 2014, between 128 Spring Street Lexington, LLC and the Seller for the area of 11,077 square feet in the Ledgemont Research Center building complex located at 128 Spring Street, Lexington, Massachusetts. As per the Lease Agreement, the rental expense for the premises would increase by a dollar per square foot annually, starting from $26.75 in the first year of the lease term and will reach $29.75 in the last year of the lease term. Since, the remaining useful life of the Lease Agreement was 1.5 years, we considered $29.75 per square foot as the rent for the forecast period ending on June 30, 2018.

GOODWILL

RNA considered the compensation, recruiting, training, and interview costs related to the assembled workforce (the "Assembled Workforce") based exclusively on information provided by Management.

Liabilities

The liabilities include the following Contingent Payments:

(i) Royalty Payments: As discussed in the "Deal Overview" section above, the Company is required to pay Royalty Payments to Shire for the IPR&D Assets;
(ii) Milestone Payments: Milestone payments include the Sales Milestone Payment and the Approval Milestone Payments (collectively "Milestone Payments"), as discussed in the "Deal Overview" section above; and
(iii) Anti-Dilution Liability: The Contingent Payments also includes anti-dilution liability of the Company related to the issue of 6,644,384 shares related to Shire - Tranche II, as discussed in the "Deal Overview" section above. Refer to the "Anti-Dilution" section below for further details on the anti-dilution liability.

MIT Agreement

Since the patent rights licensed under the MIT Agreement relate primarily to the OTC Program, we have considered payments related to the MIT Agreement to be applicable only to the OTC Program. Based on the terms of the Agreement, the Company will pay a 2.0% royalty on net sales, license maintenance fees, and certain milestone payments, based on the milestones mentioned in the MIT Agreement. The license maintenance fees for the first year will be $75,000, and it will increase by $25,000 after every two-year anniversary of the MIT Agreement. It will remain constant from January 1, 2021, at $200,000 per year. The one-time license issue fee of $75,000 has already been paid, and as such, we have not considered that expense for this analysis.

Below are the milestone payments that the Company may be required to pay to MIT:

(i) $250,000 – on acceptance of an IND application;
(ii) $450,000 – on dosing of the second patient in Phase II clinical trials;
(iii) $675,000 – on dosing of the second patient in Phase III clinical trials; and
(iv) $1,250,000 – on first commercial sale.

These payments were adjusted in the MPEEM analysis of the OTC Program as discussed in the "IPR&D Assets – Details" section below. Refer to Exhibit C.3 for further details.

Assets Excluded in the Analysis

We identified the following assets but did not value them separately. RNA estimated that the following assets would not have material value.

NON-COMPETE AGREEMENTS

Based on the information provided and discussions with Management, RNA estimated that there will be no material value attributable to the non-compete agreements due to the low probability of any of the key employees leaving to compete and no or minimal loss to the Company if they left to compete. We believed that this was reasonable given that the MRT Platform is at an early stage of development and that it is

expected to take many years before a product ultimately reaches the market. Even competitor products (other mRNA technologies) would require many years to complete clinical trials and enter the market. RNA noted that there could certainly be some delays, disruptions, or other issues if employees resigned/retired, but the non-compete agreements do not prevent such situations. As such, we have not valued non-compete agreement separately.

OTHER AGREEMENTS

RNA also considered, but did not value separately, various agreements with third-parties. Aside from the MIT agreement that was included in our analysis, none of the other agreements were deemed material or appropriate to include, such as the following:

(i) Agreement with Acuitas, other MIT agreements, and agreements with Cystic Fibrosis Foundation Therapeutics, Inc.: These agreements have effectively expired/are irrelevant as they do not pertain to existing or future development programs;

(ii) Shire – Ethris GmbH agreement: This agreement was terminated prior to the Transaction; and

(iii) Manufacturing agreement with F. Hoffmann-La Roche AG and other consultant agreements: These agreements generally carry market rate terms based on discussions with Management.

Industry Overview

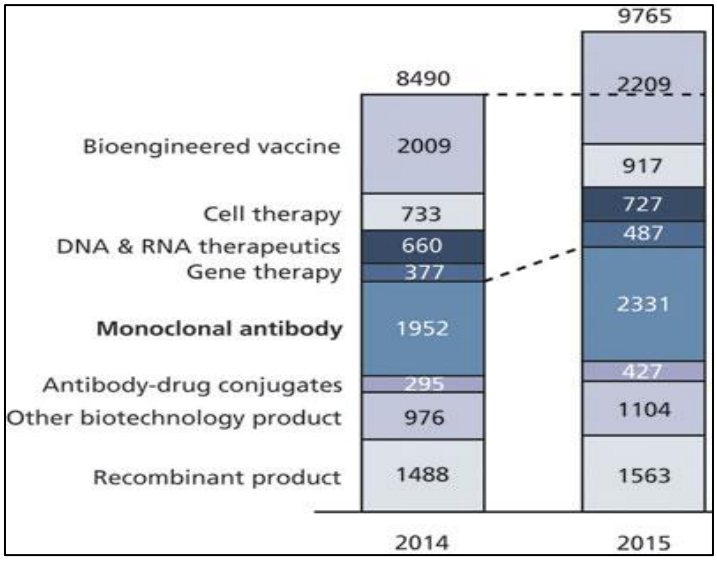
OVERVIEW

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 the RNA-based therapeutics market, with a focus on treatments for CF and OTC, as well as a discussion of the competitive environment in that space.

RNA-based Therapeutics Market

RNA-based therapeutics target the treatment of diseases such as diabetes, cancer, tuberculosis, and some cardiovascular conditions. There is currently a great deal of money being put into this relatively new class of therapeutics and vaccines, which is projected to grow 12.0% in 2016 and reach $1.2 billion by 2020. The 2015 R&D biotech pipeline is shown in the adjacent figure. There are more than 700 nucleic acid-based therapeutics (DNA and RNA) in the pipeline and more than 60.0% of the nucleic acid-based therapeutic pipeline is in preclinical development. Approximately 35.0% of such pipeline is focused on oncology. Approximately 160 companies and 65 academic institutes are developing RNA-based therapeutics.[11]

Figure 3: R&D Biotech Pipeline Expansion



[11] Source: Article published in July 2016, "Bioprocessing Technology Trends of RNA-Based Therapeutics and Vaccines", http://www.biopharminternational.com/bioprocessing-technology-trends-rna-based-therapeutics-and-vaccines.

RNAi is a natural post-transcriptional process of gene silencing involving short strands of nucleic acids. It is a regulatory process that cells utilize to silence and/or inhibit gene expression through the destruction of specific mRNA molecules. One of the major advantages of RNAi is that it enables sequence specific knockdown of a target gene.[12] According to a report by Grand View Research, Inc., the global antisense and RNAi therapeutics market is anticipated to reach $4.6 billion by 2022. Technological development in the drug delivery technology based on liposome and nano biotechnology is expected to provide the market with a high potential to grow due to enhancement in the adoption of gene silencing therapeutic product models.[13]

Cystic Fibrosis Market

According to MarketResearchReports.biz, the respiratory disorders market is forecast to rise from a value of $28.1 billion in 2015 to $46.6 billion in 2022, at a CAGR of 7.5%. The treatment landscape for respiratory disorders has traditionally been dominated by small molecule therapies that aim to treat the disease symptoms, rather than the cause. This means that treatment options can be diverse in terms of their targets and mechanisms of action. This is most notable in CF, where patients may be prescribed a combination of drugs from several treatment categories, including mucolytic agents, bronchodilators and antibiotics. Although the more traditional non-specific symptomatic therapies continue to have a strong presence in the respiratory disorder pipeline, the pipeline also contains promising targeted biologic therapies, which reflects their growing prominence in this therapy area. Overall, 908 products are currently in active in development for respiratory disorders.[14] Per a report by Visiongain, the global CF market is expected to grow at a CAGR of 16.7% from 2016 to 2021, to reach $7.7 billion.[15]

OTC/UCD Market

UCD result from defects in the metabolism of waste nitrogen from the breakdown of protein and other nitrogen-containing molecules.[16] OTC deficiency is a rare X-linked genetic disorder characterized by complete or partial lack of the OTC enzyme. OTC is one of the six enzymes that play a role in the break down and removal of nitrogen the body, a process known as the urea cycle.[17] OTC deficiency can occur as a severe neonatal-onset disease in males (but rarely in females) and as a post-neonatal-onset (partial deficiency) disease in males and females.[18] The incidence for UCD in the US is one patient for every 35,000 births, presenting about 113 new patients per year across all age groups. The incidence for OTC in the US is one patient for every 56,500 births, estimated based on the overall incidence of UCD.[19]

[12] Source: Article published in November 2016, "RNAi Therapeutics Market, 2015 - 2030", http://www.prnewswire.com/news-releases/rnai-therapeutics-market-2015---2030-300357424.html.
[13] Source: Article, "Antisense and RNAi Therapeutics Market Will Grow To $4.58 Billion By 2022: Grand View Research, Inc.", http://www.medgadget.com/2016/05/antisense-and-rnai-therapeutics-market-will-grow-to-4-58-billion-by-2022-grand-view-research-inc.html.
[14] Source: News Release, "Global Respiratory Disorders Market Forecast to Reach $46.6 Billion By 2022 - Current Clinical and Commercial Landscape: MarketResearchReports.biz", https://globenewswire.com/news-release/2016/09/14/871840/0/en/Global-Respiratory-Disorders-Market-Forecast-To-Reach-46-6-Billion-By-2022-Current-Clinical-and-Commercial-Landscape-MarketResearchReports-biz.html
[15] Source: Article, "Global Cystic Fibrosis Therapeutics Market 2016-2026", http://www.marketwatch.com/story/global-cystic-fibrosis-therapeutics-market-2016-2026-2016-11-03-11203415.
[16] Source: https://www.ncbi.nlm.nih.gov/books/NBK1217/.
[17] Source: https://rarediseases.org/rare-diseases/ornithine-transcarbamylase-deficiency/.
[18] Source: https://www.ncbi.nlm.nih.gov/books/NBK154378/.
[19] Source: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4364413/.

COMPETITION

The Company and Shire MRT face competition from other companies in the mRNA therapeutics market like Moderna Therapeutics, CureVac, BioNTech, PhaseRx, Inc., Arcturus Therapeutics, and Ethris.[20]

[20] Source: Information provided by Management.

Valuation Methodology Overview

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

VALUATION THEORY

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

(i) Cost Approach ("Cost Approach");
(ii) Income Approach ("Income Approach"); and
(iii) Market Approach ("Market Approach").

Within each category, a variety of methodologies exist 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.

Typical methods of the Cost Approach used to value an intangible asset include the following:

REPRODUCTION COST METHOD

This method contemplates the construction (or purchase) of exact replica of the subject asset. This method assumes the fact that the materials, quality of work, and technology used in the original subject asset are still available in the current scenario. As such, reproduction cost does not consider either the current market demand for or the current market acceptance of the subject intangible asset.

REPLACEMENT COST METHOD

This method contemplates the cost to recreate the utility of the subject intangible asset that serves the same purpose as the original subject asset but in a form that is not an exact replica of the subject asset. In this method, the replacement cost new is determined and reduced for depreciation of the asset. In this context, depreciation has three components: (i) physical deterioration, (ii) functional obsolescence, and (iii) economic obsolescence. Physical deterioration is impairment to the condition of the asset brought about by – wear and tear, disintegration, use in service, and/or the action of the elements. Functional obsolescence is the impairment in the efficiency of the asset brought about by such factors as overcapacity, inadequacy, or change in technology that affect the asset. Economic obsolescence is the impairment in the desirability of the asset arising from external economic forces, legislative enactment, or changes in supply and demand relationships.

RESIDUAL METHOD

The residual value method provides an estimate of the salvage value of an asset. That is, it represents the amount of value that the owner of an asset can expect to obtain when the asset is dispositioned. It is the measurement of the net income that an investment earns above the threshold established by the minimum rate of return assigned to the investment. It can be used to estimate the value of a business or a particular asset.

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. Other commonly used methods used for intangible assets valuation within the Income Approach are described below in detail:

MULTI-PERIOD EXCESS EARNINGS METHOD ("MPEEM")

This method is used in cases where there is an identifiable stream of cash flows associated with more than one asset. An MPEEM may provide reasonable indication of the value of a specific intangible asset. Under this method, the value of an intangible asset equals the present value of the incremental after-tax cash flows attributable only to the subject intangible asset, after making adjustments for the required return on and of (when appropriate) the other assets – working capital, fixed assets, and other intangible assets – required to produce the subject intangible asset cash flows. The remaining cash flow stream, or excess earnings, is attributable to the subject intangible asset being valued. An appropriate discount rate is then selected and the present value of the excess earnings stream is derived to yield the value of the subject asset.

The adjustments for the other assets are made by calculating and applying the contributory asset charges. Using guidance from the Appraisal Foundation's Best Practices for Valuations in Financial Reporting – The Identification of Contributory Assets and the Calculation of Economic Rents, contributory asset charges were utilized in the valuation of the Company's most valuable intangible asset.

Contributory assets are defined as assets that are used in conjunction with the subject intangible asset in the realization of prospective cash flows associated with the subject intangible asset. Assets that do not contribute to the prospective cash flows associated with the subject intangible asset are not contributory assets. For example, a certain amount of real property (land and buildings) may be necessary to support the cash flow attributable to a subject intangible asset. Alternatively, land held by an entity for investment (a non-operating asset) would not be appropriate to include as a contributory asset if the land is not necessary for, or expected to contribute to, the generation of the prospective cash flows of the subject intangible asset.

RELIEF FROM ROYALTY METHOD ("RFR")

The premise of this method is that the ownership of the subject intangible asset relieves the owner of the need to license the asset from a third party. Thus, by owning the intangible asset, the owner avoids the royalty payments required to license the asset. A critical element of this method is the development of a royalty rate that is comparable to ownership of the specific intangible asset. Under this method, value is estimated by discounting the royalty savings as well as any tax benefits related to ownership to a present value using an appropriate discount rate.

INCREMENTAL INCOME METHOD

Various incremental income methods are used to estimate the value of an intangible asset based on a comparison of the prospective revenues or expenses for the business or the intangible asset with and without the subject intangible asset in place. Under these methods, value is estimated by discounting the cash flow differential as well as any tax benefits related to ownership to a present value using an appropriate discount rate.

rNPV METHOD

In a typical license contract valuation, the asset is valued by estimating present value of future cash flows adjusted for probability of technical success ("POTS"). Such cash flows include upfront payments, milestone payments, royalties and adjusted for company's share of R&D and other expenses. License contract is valued from both perspectives, the licensee's and the licensor's. When we estimate the cash flows for this method, we need to understand the perspective of valuation and accordingly consider upfront payment, milestone payments and royalties as income or expense. Accordingly, we also need to factor the additional risk of achieving the set research targets; since the commonly used Capital Asset Pricing Model ("CAPM") is not capable of capturing this risk. Hence, we adjust the estimated cash flows for POTS and arrive at the NPV of the cash flows.

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.

Valuation Analysis

RNA applied the following valuation methodologies to estimate the fair value of the Subject Interest as of the Valuation Date:

(i) IPR&D Assets – MPEEM (also utilizing the rNPV Method) under the Income Approach;
(ii) Royalty and Milestone Payments – rNPV Method under the Income Approach;
(iii) Anti-Dilution Liability – NPV Method under the Income Approach;
(iv) Lease Agreement – Incremental Income Method under the Income Approach; and
(v) Assembled Workforce – Replacement Cost Method under the Cost Approach.

MPEEM – IPR&D ASSETS

The MPEEM aggregates the present value of the incremental risk-adjusted after-tax cash flows attributable only to the subject intangible asset as of the Valuation Date. The IPR&D Assets were determined to be the key assets acquired, and as such, RNA relied on the MPEEM in determining fair value indication for the IPR&D Assets. Furthermore, considering the nature and the stage of development of the IPR&D Assets, we applied the rNPV Method to calculate the cash flows within the MPEEM.

IPR&D Assets – Overall Methodology

The MPEEM methodology applied here involves the following key components:

Table 2: Cash Flow Structure for MPEEM

Cash Flow Structure
Particulars
Peak Sales
Multiply: Penetration Curve
Net Revenues
Less: Expenses
Unadjusted Cash Flows
Less: Taxes
After Tax Cash Flows
Less: Contributory Asset Charges
Net After Tax Cash Flows
Multiply: Cumulative POTS

Probability Adjusted Cash Flows
Multiply: Discount Factor
Present Value of Cash Flows
Add: Tax Amortization Benefit
Concluded Value

The key components of the cash flow structure and the related assumptions are described below:

NET REVENUES
To estimate the net revenues for the IPR&D Assets, RNA considered the peak sales value, patent life, and penetration curve associated with each of the IPR&D Assets.

PEAK SALES
RNA determined the peak sales of the CF Program and OTC Program for their respective therapeutic area based on forecasts from GlobalData, EvaluatePharma and discussions with Management. We have estimated peak sales of the MRT Platform Programs to be between those of the CF Program and the OTC Program (but closer to the OTC Program), given possible areas of pursuit and peak sales for typical gene therapy/mRNA type orphan blockbuster drugs.

Regarding the CF Program, RNA noted that there are several CF products in market with only a small subset generating or expected to generate significant sales. Some of the higher selling CF products treat symptoms, and some can only be prescribed to a subset of patients with the disease (such as patients with a specific gene mutation). Although it is far too early to tell how the CF Program will ultimately work and be prescribed to patients, it is reasonable to expect that it could serve a larger CF patient population than current treatments and generate significant sales given the perceived potential of mRNA therapies.

Regarding the OTC Program, RNA noted that there are no OTC deficiency products in market, and only a handful of UCD products. As such, RNA considered peak sales potential to be in line with typical blockbuster biopharmaceutical products as there is significant perceived potential for mRNA therapies and there is an unmet need for patients with OTC deficiency.

RNA also considered epidemiology data (worldwide prevalence and incidence rates), and average annual cost of therapy for CF, OTC and orphan diseases to corroborate the reasonability of the peak sales for the CF Program, OTC Program and the MRT Platform Programs, respectively. We noted that the annual cost of therapy for CF drugs was $250,000, and for high-end UCD drugs ranged from $250,000 to $290,000. The annual cost of therapy for orphan drugs ranged from $150,000 to $600,000. Furthermore, we also looked at the patient base covered by some of the major marketed drugs and noted that only a small proportion of the total patient population was covered by these drugs. As such, there is a potential for the IPR&D Assets to generate significant sales.

PENETRATION CURVE

RNA considered an S-curve based market penetration for all of the IPR&D Assets, assuming 5 years to peak, and 8.0% growth rates in revenues after peak based on the trends for price increases of similar drugs in the market. Refer to Exhibit H.2 for the calculation of penetration curves and Exhibit H.9 for the research study on industry trends for years to peak. This study examines the US sales trajectories (in extended units) of 70 prescription drugs approved by the FDA from 2000 to 2002, corrected to account for population growth and normalized to peak unit sales. Based on the study, the drug sales post-launch generally progress along an S-shaped curve. As such, our assumption of S-curve based market penetration with 5 years to peak was reasonable.

EXPENSES

Based on discussions with Management, budget information provided by Shire, expense trends of the guideline public companies as described in the "Market Approach – GCM" section below, development timelines, various published studies, and our industry experience, we classified total expenses in following two categories:

(i) Development Costs: These costs include R&D expenses, manufacturing expenses, and general & administrative expenses (during development phases). As noted above in the "MIT Agreement" section, we have considered expenses and payments related to the MIT Agreement for the OTC Program only; and

(ii) Commercial Costs: These costs include one-time launch expense, manufacturing expenses, general & administrative expenses, and sales & marketing expenses (post-commercialization). We estimated commercial costs as maximum of: (a) base amount, and (b) expenses as a percentage of net revenues. The base amount is the minimum expense expected to be incurred for each of the expense categories under the commercial costs.

The one-time launch expenses in the approval year reflect expenses related to promotional activities and other launch costs for the products. Typically, biopharmaceutical companies tend to spend additional capital in the launch year to help drive sales. Based on the study by Best Practices LLC, spending for launch-related activities by biopharmaceutical companies during the year their products entered the market ranged from $2.0 million to $130.0 million for both specialty and primary care products. Furthermore, on average, the study participants invested more than $35.0 million for launch-related activities; however, primary care product launches consume far greater resources than specialty launches. The primary products require two to three times the budget resources at launch year to reach the much larger population of primary care physicians.[21] Based on another report by MedAdNews, the specialty care category has a patient population in the range of 1,000 to 100,000, the cost of therapy ranges $10,000 to $100,000 annually, and the target indications are generally orphan indications. As

[21] Source: News article "New Pharmaceutical Product Launch Spend: Developing Competitive Launch and Pre-launch Budgets to Ensure a Successful Market Entry", dated September 20, 2013, http://www.prnewswire.com/news-releases/new-pharmaceutical-product-launch-spend-developing-competitive-launch-and-pre-launch-budgets-to-ensure-a-successful-market-entry-224614101.html.

such, based on these attributes the Company falls under the specialty care category.[22] Based on this analysis, the estimate of $15.0 million launch expenses was reasonable.

RNA assumed a cost of goods sold ("COGS") expense of 15.0% (85.0% gross margin) based on discussions with Management, as well as consideration of comparable company information and considering that the mRNA technology is anticipated to be relatively expensive to manufacture. RNA also studied equity analyst reports for Editas Medicine, Inc., and noted that the 15.0% COGS was considered reasonable. Furthermore, RNA assumed 40.0% selling, general and administration ("SG&A") expenses, which was in line with the third quartile of the mid-sized comparable companies presented in Exhibit H.6.

Refer to Exhibit H.6 and H.7 for details on historical expense trends of comparable companies, and refer to Exhibit H.9 for details on development costs by stage of development.

It is important to note that Shire budget information was available for certain programs for a couple of years. We leveraged that information to develop preclinical and Phase I expense estimates. For such early stage programs, especially in a technology area that is at an early stage, there is little to no precedent for later-stage clinical trial costs, as no companies/programs have yet entered those stages. As such, we considered ratios and metrics based on typical development costs for therapeutics in general, and in a few cases focused specifically on other CF programs noting that study designs may vary given different technologies and mechanisms of action.

CALCULATION OF ESTIMATED TAXES

The tax rate applied herein was based on discussions with Management, RNA's observations of tax rates for companies similar to the Company and a blended rate for the primary markets in which the Company is expected to operate. RNA generally considers a 40.0% tax rate for US corporations as the marginal corporate tax rate (on a conservative basis) noting that the rate in the US generally ranges from 38.0% to 39.0%, and varies by state. The tax rates for the EU and rest of the world are lower, typically in the range of 30.0%. Based on discussions with Management and our experience with other high-end biopharma products, the US and the major EU countries would be the primary markets for the Company. As such, the 35.0% tax rate applied was reasonable based on a blended basis of the tax rates mentioned above.

CONTRIBUTORY ASSET CHARGES ("CACs")

In order to estimate the fair value of the IPR&D Assets, we subtracted the CACs from the after-tax cash flows. As such, we applied the charge related to the Assembled Workforce, net working capital and fixed assets. The charge related to the Assembled Workforce was estimated as of the Valuation Date and then projected on the basis of the growth rate for operating expenses for the respective year. We considered the charge related to the Assembled Workforce and working capital for the years 2028 through 2036 only, since all the IPR&D Assets were commercial in this period.

[22] Source: Report "Launch success in specialty medicine" dated August 2009, http://heritagepartners.com/wp-content/uploads/2015/12/60780_wrmw2.LaunchSpecialtyMedicine.MedAdNews-2-1.pdf.

The charge related to fixed assets was forecast both as a return of the fair value of the fixed assets in each year of the projection ("*return of*"), which is considered as a recoupment of the original investment amount, as well as a pure investment return charge ("*return on*"). The *return of* charge was calculated as the annual depreciation for both the existing fixed assets and the annual projected capital expenditures, whereas the *return on* charge was calculated for each year based on the average fixed assets balance, considering a 5.0% premium on the post-tax cost of debt (a total charge of 8.1%).

Furthermore, the remaining useful life of the Lease Agreement was only 1.5 years, which would end well before the Company expects to generate any revenue for any of the IPR&D Assets. As such, we have not considered the CACs for the Lease Agreement and the related tenant improvements. Refer to Exhibit C.7 for the calculations of CACs.

POTS

POTS reflect the chance that a project entering a development phase reaches the next phase. Success rates are assessed per phase and will depend on the indication once the drug is in clinical development. There are also differences between the success rates of chemical and biological drugs. POTS for the IPR&D Assets were considered based on various published studies and FDA guidance. Refer to Exhibit H.1 and Exhibit H.10 for further details.

DISCOUNT RATE

RNA estimated discount rate to be applied to the CF Program and the OTC Program equal to the weighted average cost of capital ("WACC'). We estimated a 0.25% premium to the WACC as an appropriate discount rate to be applied on MRT Platform Programs, as the MRT Platform Programs are still in the discovery stage (or have not yet been identified, but are expected to be in the future given the intended use of the MRT Platform) and specific indications and mechanisms have not yet been identified, which would make it modestly riskier than the CF Program and the OTC Program. Furthermore, the MRT Platform Programs would also require additional capital to develop (over and above the Company's Series C financing) since they are at a very early stage of development. As such, we applied a slightly higher discount rate to the MRT Platform Programs.

TAX AMORTIZATION BENEFIT ("TAB")

IRC Section 197 requires that intangible assets be amortized over a 15-year period. As such, RNA added the present value of TAB to the present value of cash flows for each of the IPR&D Assets.

DRUG DEVELOPMENT TIMELINES

Below are the primary stages in the development of drugs[23]:

(i) Drug Discovery: Drug discovery, following basic research, can in most cases be broken down into four phases: Target identification, target validation, lead identification, and lead optimization.

[23] Source: "Valuation in Life Sciences", Bogdan and Villiger.

(ii) Clinical Development: Clinical development consists of the following sub-phases:

a. Preclinical Testing: Once a lead compound is selected, its safety has to be tested in animals before human testing takes place. Preclinical tests in animals are designed to study the effect of the drug and its metabolites in the living organism. Preclinical studies are needed to see if the drug can safely be tested in humans. The time for preclinical testing usually ranges from ten to twelve months.

b. Phase I trials: Phase I is the first phase of drug testing in humans. Phase I is typically conducted in a small group of healthy volunteers (20-80) and is designed to evaluate safety, dose range and side effects. The same questions as in preclinical testing are now assessed in humans. Absorption of the drug (bioavailability) into the blood stream is studied, as well as the mechanism of action. Furthermore, the safety profile of the drug is monitored and side effects are carefully studied and documented. A license, which is granted upon study of preclinical data, is required to start human drug trials. In the US, clinical trials cannot start prior to IND approval. On average, drugs undergo Phase I clinical trials over a period of eighteen to twenty-two months.

c. Phase II trials: In Phase II trials, the drug is studied in a group of people (100-300) having the condition to be treated. Sometimes Phase II studies are broken into separate components. Clinical Phase IIa studies are small studies to define the dose while Phase IIb studies are designed to prove effectiveness. The goal of Phase II trials is to show the proof-of-concept of the drug (i.e., that it is safe and effective in the treatment of the target disease). On average, drugs undergo Phase II clinical trials over a period of twenty-four to twenty-eight months.

d. Phase III trials: The drug is tested in a large group of patients (500-20,000) with the condition to be treated. The main aim of this phase is to confirm the effectiveness of the treatment, to disclose any side effects, and to establish the right dosage. On average, drugs undergo Phase III clinical trials over a period of twenty-eight to thirty-two months.

(iii) Approval: A company files a new drug application ("NDA") after successful clinical trials (or a biologics license application ("BLA"), as appropriate). The FDA or European Medicines Agency ("EMA") reviews the data and decides if the company is granted a marketing approval. Possibly, the regulatory authority asks for further clinical trials or even rejects marketing approval. The time for reviewing the drug ranges from sixteen to twenty months.

RNA determined the development timelines for the IPR&D Assets based on discussions with Management, the current stage of development (as mentioned in the "Assets/Liabilities Overview" section above) of the IPR&D Assets, and industry standards for similar drugs. Refer to Exhibits H.9 to H.11 for various published studies, related clinical studies, and FDA guidance on drug development timelines.

In general, timelines for the OTC Program and the MRT Platform Programs were estimated to be consistent as these products would likely be orphan disease treatments, which tend to require smaller study sizes (that ultimately make timelines shorter/faster). For the CF Program, we estimated that timelines may be longer based on our observations of timelines for other CF products in the market.

REMAINING USEFUL LIFE

Under the Patient Protection and Affordable Care Act ("PPACA"), and Biologics Price Competition and Innovation Act ("BPCIA"), new biologics are eligible for 12 years of market exclusivity, during which time the FDA cannot approve any biosimilar drugs. As such, we have considered the period from commercialization to loss of exclusivity as 12 years. Furthermore, based on the discussions with Management, and given that the sales of the products may decline dramatically after patent expiration, we have assumed no sales after loss of exclusivity. Based on this commercialization period and the development timelines as discussed in the "Drug Development Timelines" section above, RNA estimated useful life for each IPR&D Asset considering the development timelines and the period of commercial exclusivity of 12 years, and on an average determined a useful life of 20 years for each IPR&D Asset. We acknowledge that many factors can ultimately influence the economic life of a biopharmaceutical product, but given the early stage of development for the IPR&D Assets we believe the estimate herein is reasonable.

VALUE CONCLUSION

RNA estimated the value of each of the IPR&D Assets based on the structure described in Table 2 above and considering the above assumptions. RNA applied the mid-period discounting convention, and developed projections starting in 2017 considering the proximity of the Valuation Date to the end of 2016. Based on our discussions with Management and our review of the expected costs for the Transaction, there was no information breaking out the costs during the last week of December 2016, and no meaningful development activity/costs were expected in that week (primarily given holidays).

Considering the early stage of development of the IPR&D assets, there may be a range of peak sales possible for a given IPR&D Asset. As such, based on discussions with Management, observation of peak sales of marketed and pipeline products, and estimates of the expected market size for the various indications, RNA determined a range of peak sales for each IPR&D Asset, and assigned a probability to each. Also, given that the IPR&D Assets are at an early stage of development, for each of the IPR&D Asset, RNA performed a sensitivity analysis by estimating different peak sales values and assigning the corresponding probabilities to each of the peak sales scenarios. RNA assigned the highest probability to the peak sales number which we estimated as most likely based on the above considerations, and created a distribution around the peak with the mid-case peak sales at 45.0% probability, the next two cases (one on either side of the mid-case) probability adding up to 45%, and the final 10% probability split amongst the outer ends of the ranges. We had probabilities slightly skew above the mid-case considering the factors supporting a higher potential for outperforming the mid-case, such as growth in the market place, inflation and other factors.

Thereafter, RNA calculated and concluded the probability weighted average value for the respective IPR&D Assets. These analyses intended to give us a sense of the sensitivity to valuation under different assumptions (including expectations around achieving certain sales milestones) noting that the IPR&D Assets are at a very early stage of development, very little information exists regarding the commercial

viability of the underlying products, and the mRNA market is at a very early stage as well. Refer to Exhibits C.1 through C.6 for further details.

IPR&D Assets – Details

CF PROGRAM

To determine the value associated with the CF Program, RNA considered the following key assumptions to develop the rNPV analysis:

(i) Net Revenues: Net revenues were forecast based on the WW sales estimates consisting of peak sales and penetration curve assumptions. Refer to Exhibit H.3 for further details; and

(ii) Phase of Development: The CF Program is currently in the preclinical stage, is expected to initiate clinical trials in 2018, and is expected to reach the market in 2027 given estimated development timelines.

RNA performed a sensitivity analysis of peak sales for the CF Program to calculate and conclude to the probability weighted average value of the CF Program at $42.3 million. Refer to Exhibit C.2 for details on the rNPV analysis, and Exhibit B.1 for rNPV assumptions.

OTC PROGRAM

To determine the value associated with the OTC Program, RNA considered the following key assumptions to develop the rNPV analysis:

(i) Net Revenues: Net revenues were forecast based on the WW sales estimates consisting of peak sales and penetration curve assumptions. Refer to Exhibit H.4 for further details;

(ii) Phase of Development: The OTC Program is currently in the preclinical stage, is expected to initiate clinical trials in 2018, and is expected to reach the market in 2025 given estimated development timelines; and

(iii) Payments related to MIT Agreement: In addition, as described in the "Assets/Liabilities Overview" section above, we have subtracted the payments related to the MIT Agreement.

RNA performed a sensitivity analysis of peak sales for the OTC Program to calculate and conclude to the probability weighted average value of the OTC Program at $18.6 million. Refer to Exhibit C.3 for details on the rNPV analysis, and Exhibit B.2 for rNPV assumptions.

MRT PLATFORM PROGRAMS

To determine the value associated with the asset, RNA considered the following for purposes of developing the rNPV analysis:

(i) Net Revenues: As discussed above, net revenues were forecast based on the peak sales for typical gene therapy/mRNA type orphan blockbuster drugs, as well as general penetration curve assumptions; and

(ii) Phase of Development: Based on discussion with Management, the Company is expected to initiate lead optimization and preclinical studies for the Pipeline Program #1, the Pipeline Program #2, and the Pipeline Program #3 in 2017, 2018, and 2019, respectively, and they are expected to commercialize in 2026, 2027 and 2028 respectively. The timelines essentially mirror those for the OTC Program with development starting one year apart for each pipeline program. Furthermore, we considered a duration of 2 years to complete the lead optimization and preclinical studies considering that the MRT Platform Programs are at an earlier stage as compared to the CF Program and the OTC Program.

RNA performed a sensitivity analysis of peak sales for the MRT Platform Programs to calculate and conclude to the probability weighted average value of the Pipeline Program #1, the Pipeline Program #2, and the Pipeline Program #3 at $18.0 million, $15.2 million and $12.8 million, respectively. Refer to Exhibits C.4 through C.6 for details on the rNPV analysis, and Exhibits B.3 through B.5 for rNPV assumptions.

RNA also gauged details on various comparable preclinical platform deals to ascertain the reasonability of the value concluded above. Other transactions in the market for early stage technology platforms with the potential to generate clinical development candidates tend to consist of meaningful upfront payments with significant contingent payments (milestones and royalties) if products are ultimately successful. For the platform itself, RNA considered the upfront component of these comparable transactions as a reasonable benchmark for the value of the MRT Platform (essentially representing the price others have paid to access interesting technology to develop clinical candidates). Refer to Exhibit H.5 for further details.

RNPV APPROACH – ROYALTY AND MILESTONE PAYMENTS

For purposes of this analysis, RNA relied on the rNPV method to determine the valuation indication for the Royalty Payments and Milestone Payments.

Royalty Payments

Based on the Asset Purchase Agreement and as explained in the "Deal Overview" section above, the Company is required to pay a 4.0% royalty on net revenues for each of the IPR&D Assets to Shire until the loss of exclusivity. RNA applied the Royalty Payment Rate to the net revenues for each of the IPR&D Assets, and also considered a tax benefit since the Royalty Payments would reduce the tax liability of the Company to calculate net royalty payments ("Net Royalty Payments"). We then adjusted the Net Royalty Payments for POTS (consistent with the respective POTS applied for each of the IPR&D Assets) to calculate the probability adjusted net royalty payments ("Probability Adjusted Royalty Payments").

DISCOUNT RATE

RNA considered 15.0% as an appropriate discount rate to be applied on the Royalty Payments, based on the cost of debt (venture debt or another similar structured debt) issued to similar companies in the life sciences industry. RNA noted that the range of cost of debt was 7.3% to 11.5% for similar companies with

an average cost of debt of approximately 10.0%. Refer to Exhibits H.14 through H.16 for further details. However, such cost of debt noted above does not include considerations apart from interest rates, such as warrant coverage, administrative charges, final payment fees and origination fees. Such data is not usually summarized publicly, though in our experience these items would add the equivalent of a few percentage points to the interest rate. RNA also considered credit risk associated with these payments, based on the credit risk premium for large pharmaceutical companies, which was 1.5%, on average. Refer to Exhibits H.17 for further details.

VALUE CONCLUSION

RNA applied the discount rate mentioned above to the Probability Adjusted Royalty Payments to determine the present value of Royalty Payments. Similar to the IPR&D Assets, RNA applied the mid-period discounting convention and also performed a sensitivity analysis by estimating different peak sales and assigning the corresponding probabilities to each of the peak sales scenarios. Thereafter, RNA calculated and concluded to the probability weighted average value of the Royalty Payments. Refer to Exhibits D.1 through D.6 for further details.

Milestone Payments

Based on the Asset Purchase Agreement and as mentioned in the "Deal Overview" section above, the Company is required to make Sales Milestone Payment and Approval Milestone Payments to Shire. RNA calculated the fair value of Approval Milestone Payments and Sales Milestone Payment as mentioned below. We considered a tax benefit from the Milestone Payments to calculate net milestone payments as the Milestone Payments would reduce the tax liability of the Company.

APPROVAL MILESTONE PAYMENTS

As explained in the "Deal Overview" section above, the Approval Milestone Payments will be triggered on the occurrence of each of the following: (i) first commercial sale of the CF Program, and (ii) first commercial sale of any of the other IPR&D Assets. As such, we have calculated Approval Milestone Payments for the CF Program and the other IPR&D Assets. We considered the Approval Milestone Payments of $35.0 million payable on the approval of the CF Program in 2027, and $10.0 million each, payable on the approval of the OTC Program in 2025, Pipeline Program #1 in 2026, Pipeline Program #2 in 2027, and Pipeline Program #3 in 2028. We then applied POTS (consistent with the POTS applied for the IPR&D Assets) and the discount factor as discussed below to calculate the present value of the Approval Milestone Payments. Refer to Exhibits D.1 through D.6 for further details.

SALES MILESTONE PAYMENT

Based on the Asset Purchase Agreement, the Sales Milestone Payment will be triggered upon the first IPR&D Asset achieving annual net revenues of $500.0 million. As such, considering that the Sales Milestone Payment may be triggered for any of the IPR&D Assets, we estimated the probabilities for each of the IPR&D Assets achieving the Sales Milestone. Based on the current development timeline of the IPR&D Assets and to a lesser extent, their peak sales estimates, we have assumed the following sequence for the IPR&D Assets to achieve the Sales Milestone:

(i) CF Program;
(ii) OTC Program;
(iii) Pipeline Program #1;
(iv) Pipeline Program #2; and
(v) Pipeline Program #3.

Based on the cumulative success probability of each IPR&D Asset (estimated based on the POTS), we have calculated the probability weighted Sales Milestone Payment. Based on the sequence mentioned above, we have assumed that the CF Program will achieve the Sales Milestone and will receive the Sales Milestone Payment. If, however, the CF Program fails and the OTC Program succeeds, then the OTC Program will receive the Sales Milestone Payment. In the same manner, if both the CF Program and the OTC Program fail, the subsequent IPR&D Assets in the sequence listed above will receive Sales Milestone Payment. We have also considered a scenario, wherein all the IPR&D Assets fail and the Sales Milestone is not achieved.

POTS

The calculation of the fair value of the Sales Milestone Payment is built up such that it covers the individual success probabilities of the IPR&D Assets, and cumulative success probabilities of a particular IPR&D Asset given that the earlier IPR&D Asset(s) fails. To calculate the cumulative success probability of the IPR&D Assets, we multiplied the individual success probability by the cumulative failure probability of the earlier IPR&D Asset(s).

DISCOUNT RATE

RNA considered 15.0% as an appropriate discount rate similar to the discount rate for Royalty Payments.

VALUE CONCLUSION

Similar to the IPR&D Assets, RNA applied mid-period discounting convention and also performed a sensitivity analysis by estimating different peak sales and assigning the corresponding probabilities to each of the peak sales scenarios. Thereafter, RNA calculated and concluded to the probability weighted average value of the Sales Milestone Payment. We summed up the present value of the Approval Milestone Payments from the CF Program and the other IPR&D Assets to calculate fair value of the Approval Milestone Payments. Refer to Exhibits D.1 through D.6 for further details.

INCREMENTAL INCOME METHOD – LEASE AGREEMENT

According to the terms of the Lease Agreement, Shire currently pays $29.75 per square foot as the rental expense for occupying the property. Based on discussions with Management and the market rates for similar properties in the area, the market rental expense was considered at $35.00 per square foot for a comparable property. Refer to Exhibit E.2 for the details on the comparable market data. RNA applied the incremental income method to calculate the incremental value generated due to the lower rental expense compared to the market rates. The cash flows were then discounted at a discount rate of 8.1%. The lower discount rate is warranted considering that the cash flows related to the Lease Agreement are

more certain compared to the cash flows associated with the other intangible assets. The present value of the TAB was added to the present value of the cash flows to estimate the value of the Lease Agreement at $65,500. Refer to Exhibit E.1 for further details.

REPLACEMENT COST METHOD – ASSEMBLED WORKFORCE

RNA considered the recruiting, training, and interview costs related to the Assembled Workforce (based exclusively on information provided by Management) to estimate the value of workforce at $0.7 million. Refer to Exhibit E.4 for further details.

NPV METHOD – ANTI-DILUTION LIABILITY

Based on the Asset Purchase Agreement and as mentioned above in the "Deal Overview" section, if the Company raises Series C – Tranche II, the Company is required to issue Common Stock to Shire to maintain the its 18.0% holding on an as converted basis or 19.9% voting power in the Company. Management indicated that there is a high probability of the Company raising the Series C –Tranche II at the end of the first quarter of 2017, and as such, we calculated the number of shares to be issued to Shire in the second tranche, as calculated in the Common Stock Valuation Opinion. Furthermore, based on discussions with Management, we assigned 100.0% probability to the Series C – Tranche II raise and calculated the value of Shire – Tranche II based on the per share value of Common Stock concluded in the Common Stock Valuation Opinion. We then discounted the value of Shire – Tranche II with a 3-month risk-free rate and calculated the present value as of December 31, 2016 (noting no meaningful difference than calculating it as of the Valuation Date although we would have to interpolate risk-free rates given the few extra days beyond 3 months).

To calculate the value of Anti-Dilution Liability, we considered the difference between the value of Shire – Tranche II as of the expected raise date, and the present value of Shire – Tranche II as of December 31, 2016. Refer to Exhibit D.7 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. Notwithstanding, RNA did consider Guideline Public Companies deemed comparable to Shire MRT, a key element of the Market Approach. Refer to Exhibit G.2 and Exhibits H.6 through H.8 for details on capital structure, beta, market capitalization, enterprise value, operating metrics, net working capital and

capital expenditures requirement, and business descriptions, and for the selected Guideline Public Companies.

For the purpose of calculation of WACC (and capital structure and beta) and estimate the capital expenditure and net working capital ratios, RNA considered the following Guideline Public Companies:

(i) Ionis Pharmaceuticals, Inc.;
(ii) Horizon Pharma plc;
(iii) Alnylam Pharmaceuticals, Inc.;
(iv) Sarepta Therapeutics, Inc.;
(v) Juno Therapeutics, Inc.;
(vi) CRISPR Therapeutics AG;
(vii) Editas Medicine, Inc.;
(viii) NantKwest, Inc.;
(ix) Intellia Therapeutics Inc.;
(x) PhaseRx, Inc.; and
(xi) Dicerna Pharmaceuticals, Inc.

RNA selected these Guideline Public Companies based on consideration of the Common Stock Valuation Opinion, discussions with Management and our own research. The Guideline Public Companies include companies that develop gene therapy products (such as CRISPR Therapeutics AG and Editas Medicine, Inc.), RNA-based products (such as PhaseRx, Inc.), and companies that develop products targeting rare diseases (such as Horizon Pharma plc).

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

(i) Shire MRT's operations focus primarily on mRNA therapeutics, which is generally different from and/or less diversified than the businesses of the selected Guideline Public Companies;
(ii) Shire MRT 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);
(iii) The Shire MRT is generally smaller and at an earlier development stage than the Guideline Public Companies;
(iv) 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 Shire MRT represent a higher risk profile than the Guideline Public Companies; and
(v) Shire MRT 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.

Furthermore, RNA considered the following Guideline Public Companies to estimate the expense trends primarily related to commercial costs, since majority of the companies comparable to Shire MRT are at an early stage of development and do not provide meaningful expense trends:

(i) Regeneron Pharmaceuticals, Inc.;
(ii) Grifols, S.A.;
(iii) Alexion Pharmaceuticals, Inc.;
(iv) Vertex Pharmaceuticals Incorporated;
(v) United Therapeutics Corporation;
(vi) Opko Health, Inc.;
(vii) BioMarin Pharmaceutical Inc.;
(viii) Incyte Corporation;
(ix) Myriad Genetics, Inc.;
(x) Alkermes plc;
(xi) Emergent BioSolutions Inc.; and
(xii) BioMarin Pharmaceutical Inc.

DISCOUNT RATES

WACC

We considered WACC for estimating an appropriate discount rate for the Subject Interest. It is comprised of the following elements:

(i) Cost of Debt ("K_d"): Reflects the cost of a hypothetical senior secured loan facility;
(ii) Tax Rate ("T"): Reflects an all-in marginal corporate tax rate;
(iii) Cost of Equity ("K_e"): Reflects the required rate of return for an equity investment;
(iv) % Debt ("D/C%"): Reflects the percentage of debt in the capital structure; and
(v) % Equity ("E/C%"): Reflects the percentage of equity in the capital structure.

The WACC formula is as follows: K_d *(1-T)*D/C% + K_e *E/C%

With respect to determining the above WACC inputs, RNA noted the following:

(i) K_d:
   a. Considered the average yield on the Moody's Baa Rated Corporate Index as of the Valuation Date;
   b. Considered the cost of debt for certain companies deemed comparable to Shire MRT;

c. Considered Shire MRT's weighted-average cost of debt as of the Valuation Date; and
d. Considered discussions with Management regarding market interest rates.

(ii) T: Based on discussions with Management and RNA's observations of tax rates for companies similar to Shire MRT;

(iii) D/C% and E/C%:
a. Considered the capital structure for certain companies deemed comparable to Shire MRT;
b. Considered Shire MRT's capital structure as of the Valuation Date; and
c. Considered discussions with Management regarding the degree of financial leverage Shire MRT could reasonably bear.

(iv) K_e:
a. Considered the historical rates of return for venture capital firms, as further discussed below; and
b. Considered the CAPM, as further discussed below.

The CAPM is comprised of the following elements:

(i) Risk-Free Rate ("R_f"): Reflects a risk-free rate of return;
(ii) Beta ("β"): Reflects the sensitivity of the expected excess asset returns to the expected excess market returns;
(iii) Market Risk Premium ("R_m"): Reflects the expected return of the market;
(iv) Size Premium ("R_s"): Reflects a risk premium for small size; and
(v) Unsystematic Risk Premium ("R_u"): Reflects a risk premium for any unsystematic or company-specific risks.

The CAPM formula is as follows: $K_e = R_f + \beta^*(R_m) + R_s + R_u$

With respect to determining the above CAPM inputs, RNA noted the following:

(i) Risk-Free Rate (R_f): Considered the yields on 20-year US treasuries as of the Valuation Date;
(ii) Beta (β): Based on unlevered beta of the selected Guideline Public Companies and re-levered based on the applied capital structure;
(iii) Market Risk Premium (R_m): Based on the Duff and Phelps 2016 Valuation Handbook;
(iv) Size Premium (R_s): Based on the Duff and Phelps 2016 Valuation Handbook – decile 10; and
(v) Unsystematic Risk Premium (R_u): Based on RNA's assessment of Shire MRT's risk factors previously discussed. We ultimately applied a 0.0% premium given the high beta applied.

Based on the considerations herein, RNA estimated a WACC of 19.0%. Refer to Exhibit G.1 for further details. Furthermore, to determine the reasonability of the discount rate determined using WACC, we also studied the discount rate survey published by Avance and Biostrat. The WACC of 19.0% was within the range of the discount rates for the early- to mid-stage companies, which is reasonable considering the development stage of Shire MRT. Refer to Exhibit G.3 for further details.

Weighted Average Return on Assets ("WARA")

The WACC is generally the starting point for determining the discount rate applicable to an individual intangible asset. Premiums and discounts are applied to the company's WACC to reflect the relative risk associated with the particular tangible and intangible asset categories that comprise the group of assets expected to generate the projected cash flows. The process of disaggregating the discount rate is typically referred to as "rate stratification." The range of discount rates assigned to the various tangible and intangible assets should reconcile, on a value-weighted basis, to the entity's overall WACC.

RNA assessed the overall reasonableness of the discount rates assigned to each asset by generally reconciling the discount rates assigned to the individual assets, on a value-weighted basis, to the WACC of Shire MRT. Although goodwill is not explicitly valued by discounting residual cash flow, its implied discount rate should be reasonable, considering the facts and circumstances surrounding the transaction and the risks normally associated with realizing earnings high enough to justify investment in goodwill. Refer to Exhibit G.4 for the rates of return estimated for the different assets in the analysis. The discount rates selected for the intangible assets in conjunction with the rates selected for other assets, including goodwill, resulted in a WARA of 18.9%, in line with Shire MRT's WACC of 19.0%. Therefore, the selected discount rates assigned to the assets acquired appear reasonable.

The rate applied to the current assets, such as inventory, were generally at a premium to the after-tax US prime bank rate. The rate applied to the fixed assets, Lease Agreement and tenant improvements was generally at a premium to the after-tax cost of debt as explained in the "WACC" section above. For the intangible assets, RNA assumed the rate of return for the CF Program and the OTC Program equal to the WACC, and the rate of return for the MRT Platform Programs at a premium of 0.25%, as discussed in the "IPR&D Assets" section above. The discount rate for the Assembled Workforce was estimated at a 1.5% premium to the WACC, since it is considered riskier than the other intangible assets. The implied discount rate for goodwill, which is at 1.5% premium to the WACC, is higher than the discount rates for the identified intangible assets considering it to be the riskiest asset on the balance sheet (along with the Assembled Workforce). Refer to Exhibit G.4 for further details.

Internal Rate of Return ("IRR")

To calculate IRR, we considered the total cash flows at enterprise level. We considered the probability adjusted net revenues and expenses from the IPR&D Assets as explained in the "IPR&D Assets" section above to calculate the probability adjusted cash flows. We made adjustments to the cash flows for net operating losses ("NOL"). We considered the beginning balance of the NOL as zero since the deal was

structured as an asset deal. The NOL balances generated as a result of operating losses during the forecast period were then applied in each year of the forecast period, as available and as appropriate, to estimate the taxable cash flow. We then applied taxes to calculate cash flows after tax ("After Tax Cash Flows"). With respect to net working capital requirements and capital expenditures, RNA primarily relied on the net working capital and capital expenditure requirements of selected Guideline Public Companies. After making adjustments for net working capital and capital expenditure, we discounted the net probability adjusted after tax cash flows to determine the present value of the cash flows. As discussed above, similar to the IPR&D Assets and the Contingent Consideration, RNA applied the mid-period discounting convention to estimate the IRR.

We then subtracted the fixed assets from the present value of the cash flows to calculate the TAB for the Subject Interest. We then added TAB to calculate the total enterprise value. The discount rate was backsolved such that the total enterprise value is equal to the total purchase consideration. The implied IRR from the above calculation was 18.8%, which is in line with the WACC of 19.0% and WARA of 18.9%. Refer to Exhibit F.1 for further details.

COMPUTATION OF RESIDUAL GOODWILL AND RECONCILIATION

The goodwill was calculated by subtracting the value of the current and long-term tangible assets, as well as the fair value of the identifiable intangible assets from the enterprise value. The residual goodwill (including Assembled Workforce) was estimated at $5.3 million or approximately 4.7% of the Purchase Consideration. Refer to Exhibit A.1 for further details.

Conclusion

Based on our analysis, it is our opinion that the fair value of the Subject Interest as of the Valuation Date is reasonably stated as follows:

Table 3: Valuation Summary

VALUATION SUMMARY	(USD IN THOUSANDS)
Asset	**Value Indication**
Tangible Assets	2,416.2
Intangible Assets	
IPR&D Assets	
CF Program	42,291.4
OTC Program	18,559.0
MRT Platform Programs	45, 991.9
Lease Agreement	65.5
Goodwill	
Assembled Workforce	686.7
Goodwill	4,649.2
Concluded Fair Value of the Subject Interest	**114,660.0**
Purchase Consideration	**Value Indication**
Total Value of Shares Issued to Shire	49,539.8
Payment to MTS	2,500.0
Contingent Consideration	
Royalty and Milestone Payments	62,665.8
Anti-Dilution Rights	(42.9)
Total Contingent Consideration	**62,662.9**
Total Purchase Consideration	**114,660.0**

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

- 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 American Institute of Certified Public Accountants Statement on Standards for Valuation Services.

Sincerely,

DRAFT

Samuel Renwick, CFA

Exhibits