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Strategic Advisory Analytics

Gene Therapy - Near-term Revolution or Continued Evolution?

Part 1: Global Proprietary Data (Abbreviated Version)

Ravi Mehrotra, Ph.D.

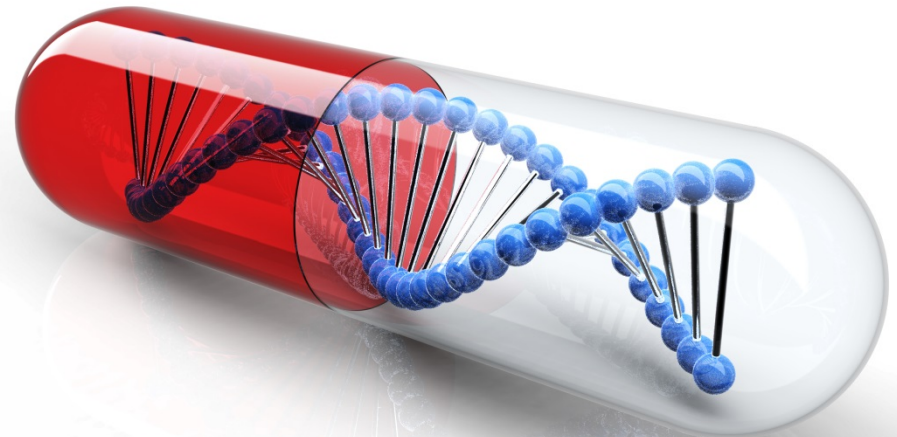
August 2017

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Gene Therapy - Near-term Revolution or Continued Evolution?

Part 1: Global Proprietary Data

□ Despite the huge investment in time and money, the first approved Gene Therapy products were not until uniQure's Glybera in July 2012, followed by Ionis' Kymriah in January 2013. Both of these pioneering products (as well as GSK's Strimvelis, approved in May 2016) were commercial non-starters.

□ 2016 was the year for the first commercially viable Gene therapies, with the September approval of Sarepta's Exondys 51 and the approval of Biogen's Spinraza in December – both of which are expected to generate >\$1bn peak sales.

This is an abbreviated version of MTS' Report:

□ Moreover, the next 24 months will see a plethora of surrogate validations for Gene Therapy in the form of potential product approvals and at least another 8 product candidates in registration-enabled clinical trials. At least 11 of these 11 near-term potential approvals have peak revenue potential of >\$1bn.

Gene Therapy - Near-term Revolution or Continued Evolution? – Part 1

□ Thus the key question is, "Are we about to witness the coming of the Gene Therapy age and a near term revolution, or will the sector still need further time to mature?" This is the topic of second section of the report.

Please contact Ravi Mehrotra for the full Report.

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□ Within this current set of reports we will attempt to answer questions such as "Is Gene Therapy on the cusp of delivering on its promise as a paradigm-shifting therapeutic class?", "Will the rules of the game for other therapeutic classes apply?", and "What will be the key metrics for predicting success in Gene Therapy?"

MTS' Strategic Advisory Analytics Report on Gene Therapy

Full Report Divided into Three Parts

Part 1 Published August '17

Part 1: Global Proprietary Data

Key valuation and performance data from MTS' proprietary database of 190 global public and private Gene Therapy companies. We also look at the late-stage pipeline of the Gene Therapy sector and put this into context of the totality of the Biopharma pipeline.

Part 2: The Gene Therapy Ecosystem

Description of the Gene Therapy space, the various technologies, and current business models, as well as analysis of the potential impact of approvals and launches on the Biopharma industry. We'll analyze the clinical, regulatory, and manufacturing hurdles that could set apart the winners from the not-so-much winners in the space.

Part 3: Conclusions for the Future of the Gene Therapy Space

Dives deeper into the unique sensitivities that each Gene Therapy approach faces in the near-term, providing key insights and potential actionables with goal of outlining a detailed roadmap to successfully navigating the coming era of Gene Therapy.

Part 2 and 3 to be published in Fall '17 and Q4'17, respectively

Summary of Previously Published Strategic Advisory Analytics Reports

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MTS | HEALTH PARTNERS
Strategic Analytics

Principled Drug Pricing Centered on Innovation and Choice: Part 1

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Strategic Advisory Analytics Report #1:

Principled Drug Pricing Centered on Innovation and Choice: Part 1

Strategic Advisory Analytics Feedback #1:

Will Drug Pricing Still be as Big of an Issue Going Forward with the New US Administration?

Strategic Advisory Analytics Feedback #2:

Unprincipled Price Rises: Where is the Value in the Unbearable Symptom?

Strategic Advisory Analytics Feedback #3:

The Frictional Cost Break-up in Drug Pricing: It's not Me, It's You

Strategic Advisory Analytics Feedback #4:

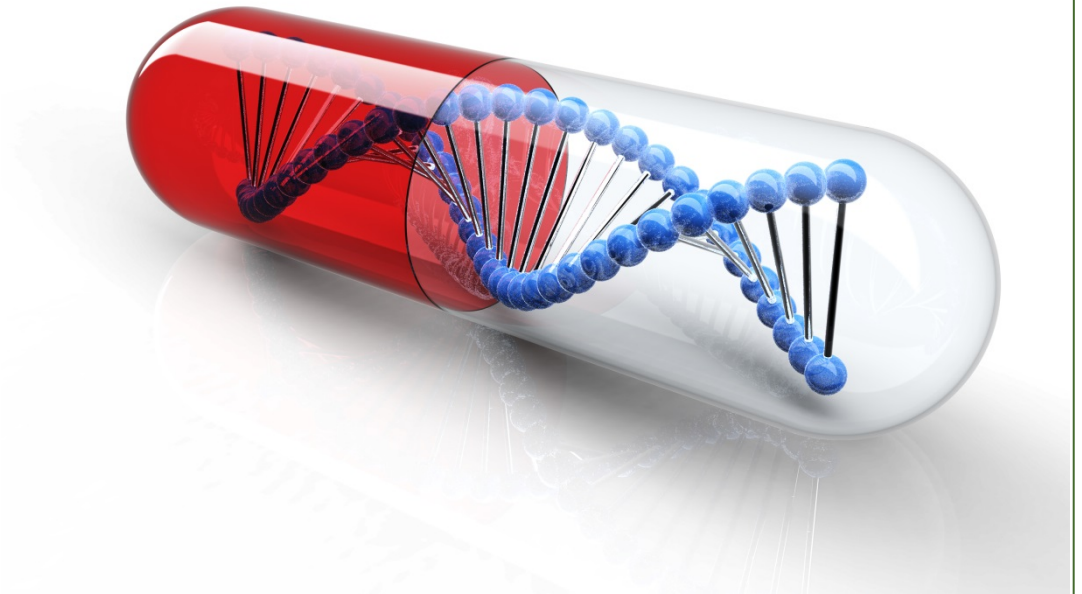
Orphan Drug Pricing – Unlikely to be Left Alone in the Current Pricing Debate

Strategic Advisory Analytics Feedback #5:

#complex drug pricing in 140 characters or less

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I. Executive Summary

Executive Summary

Gene Therapy - Near-term Revolution or Continued Evolution?

- **By implication the world thinks Gene Therapy is THE future of therapeutics....**
 - Gene Therapy companies now represent 20% and 29% by number and market capitalization, respectively, in our database of 490 precommercial public biotech companies (see slide #15).
 - This makes Gene Therapy the 2nd largest genre of companies, beating mAbs (PS: there are more than twice the number of Gene Therapy assets in clinical development than mAbs, see slide #17) and only surpassed by small molecule companies.
 - Moreover, the mean market capitalization of precommercial Gene Therapy companies (\$367mm) is greater than companies developing monoclonal antibodies (\$362mm) and significantly larger than small molecules companies (\$250mm) (see slide #16).
 - This implied enthusiasm reflects the view that Gene Therapy is the “ultimate frontier” of therapeutic technologies that could revolutionize medicine a magnitude greater than small molecules and biologics ever did.
- **It's been a long time evolving, but are we finally on the cusp of the Gene Therapy therapeutic revolution?**
 - It's been just over 25 years since the first human Gene Therapy therapeutic approach (for the history buffs amongst us, it was the 14th September 1990) and more than 1700 clinical trials evaluating Gene Therapies have been conducted in that time.

Executive Summary

Gene Therapy - Near-term Revolution or Continued Evolution?

- Despite the huge investment in time and money, the first approved Gene Therapy products were not until uniQure's Glybera in July 2012, followed by Ionis' Kynamro in January 2013. Both of these pioneering products (as well as GSK's Strimvelis, approved in May 2016) were commercial non-starters.
- 2016 was however, the year for the first commercially viable Gene Therapies, with the September approval of Sarepta's Exondys 51 and the approval of Biogen's Spinraza in December – both of which are expected to generate >\$1bn peak sales.
- Moreover, the next 24 months provide a plethora of surrogate validations for Gene Therapy in the form of potential product approvals – there are 3 products currently filed and expected to be approved by year end, and at least another 8 product candidates in registration-enabling studies with top-line data imminent. Five of these 11 near-term potential approvals have peak revenues of >\$1B.
- Thus the key question is, “Are we about to witness the coming of the Gene Therapy age and a near term revolution, or will the sector still need further time to mature, and rather we are set for continued evolution”? This is the topic of second series of MTS' “Strategic Advisory Analytics” reports (the first series was on [drug pricing](#)).
- Within this current set of reports we will attempt to answer such questions as “Is Gene Therapy on the cusp of delivering on its promise as a paradigm-shifting therapeutic class?”, “Will the rules of the game for other therapeutic classes apply?”, and “What will be the key metrics for predicting success in Gene Therapy”?

Executive Summary

Gene Therapy - Near-term Revolution or Continued Evolution?

- **The Gene Therapy space has evolved into a heterogeneous and complex set of technologies, with significantly different clinical, regulatory, and manufacturing hurdles, as well as business models**
 - The field of Gene Therapy has massively evolved scientifically into a heterogeneous and complex set of technologies since the above described first origins of "simple" Gene transfer in 1990. Many of these could easily be described as standalone technologies (or at least discrete subsets of Gene Therapy), such as mRNA based technologies, CAR-T, TCR, CRISPR, etc.
 - Concomitantly, the words "Gene Therapy" capture an incredibly wide set of approaches in the current day biopharma industry. To further increase the complexity in the Gene Therapy space, the underlying business models of the companies in the space also show a disparate range, from principally platform technology plays, through single asset plays (using “generic” technologies), to companies attempting a fast route to a “FIPCO-like” model.
 - With this inherent heterogeneity in the Gene Therapy space it's no wonder that even sector experts find the field complex, and for us mortals, the word “confusing” bubbles up.
- **MTS' method to demystifying the opportunities and treats in the Gene Therapy space**
 - The commonality of all companies within the Gene Therapy space is the use of genetic material (DNA and RNA) to treat a broad range of diseases, from monogenic disorders to multi-factorial diseases such as cancer.
 - Our central approach to demystify the totality of the Gene Therapy space has been to assign each company to one of four groups, which we call “approaches”; (1) Gene transfer, (2) Gene disruption, (3) Gene-modified cell therapy and (4) Gene editing companies.

Executive Summary

Gene Therapy - Near-term Revolution or Continued Evolution?

- **Our Gene Therapy report will be published in 3 parts**

- **Part 1** of our Gene Therapy Series, **“Global Proprietary Data”**, looks at key valuation and performance data from MTS' proprietary database of Gene Therapy companies. We also look at the late-stage pipeline of the Gene Therapy sector and put this into context of the totality of the Biopharma pipeline.
- **Part 2** of the Gene Therapy Series, **“The Gene Therapy Ecosystem”** (to be published in the Fall), will describe the Gene Therapy space in detail, highlighting the various technologies and current business models, as well as analysis of the potential impact of approvals and launches on the biopharma industry.
- **Part 3** of our Gene Therapy Series, **“Conclusions for the Future of the Gene Therapy Space”** (to be published in Q4), will dive deeper into the unique sensitivities each approach faces in the near-term, providing key insights and potential actionables with the goal of outlining a detailed roadmap for successfully navigating the coming era of Gene Therapy.
- In the same approach we took to our drug pricing work, the main aim of this report is to act as a forum for continued debate on the sector and thus feedback from both corporates and investors is welcomed (please email mehrotra@mtspartners.com).

Executive Summary

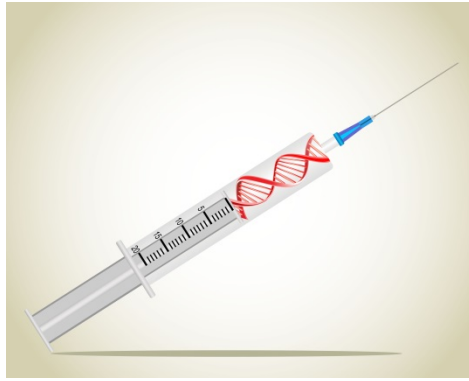
Part 1: Global Proprietary Data

- **Part 1 “Global Proprietary Data” includes key valuation and performance data from MTS' proprietary database**
 - We have created a unique and proprietary pure-play Gene Therapy database, which includes 53 public and 137 private companies.
 - We assigned each company within the database to one of four core Gene Therapy approaches: **(1) Gene transfer, (2) Gene disruption, (3) Gene-modified cell therapy and (4) Gene editing.**
- **Top 10 Key takeaways from Part 1 of our report:**
 1. **Gene transfer and Gene disruption are the leading approaches** for both public and private companies, representing 42% and 28% of public companies, and 55% and 23% of private companies, respectively (see slides #19 and #34-40).
 2. **Gene-modified cell therapy is an emerging major approach**, which represents 24% and 18% of public and private companies, respectively. Gene editing is the newest approach with 3 public and 5 private companies (see slides #19 and #34-40).
 3. In our opinion, **no single approach is likely to be the winner** per se and we see room for multiple winners in each approach. Because of the number of total companies across all approaches, we also expect there will be a significant number of not-so-much winners.

Executive Summary

Part 1: Global Proprietary Data

4. **Three of the 4 approaches** (Gene transfer, Gene disruption and Gene modified cell therapy) are expected to have an **approved product by YE'17** (see slide #20).
5. To date only 5 total Gene Therapy products are approved/marketed. However there are 11 filed/late-stage candidates in development (see slide #20), suggesting that **the next 24 months have potential to be a bellwether of long-term sector sentiment**.
6. **Valuation within approaches is bimodal**, with larger market caps attributed to those companies with potential near-term product approvals (see slide #21). Interestingly, the most mature companies are not capturing the highest valuations. The recent IPO window has produced the majority of the valuation leaders (see slide #22).
7. **The top ten Gene Therapy** products by revenue in 2022 are expected to **capture more than 50% of the entire market**, meaning near term revenue will largely benefit a few key players (slide #23).
8. Candidates in Gene transfer, Gene disruption, and Gene-modified cell therapy approaches have blockbuster potential (see slide #24), with **Hematology, Oncology and Rare disease markets most likely to be disrupted**.
9. **Sector M&A has been light** (see slides #25 and #26), suggesting large cap BioPharma is looking for justification for current valuations – larger M&A appetites may emerge as more late-stage candidates achieve approval.
10. **BD&L has been more in favor than M&A** but total deal numbers and deal values are far from overwhelming (see slide #27 and #28). Interestingly activity has been equally distributed across all Gene Therapy approaches, with large cap BioPharma showing slight preference for in-licensing Gene transfer candidates (see slide #29)

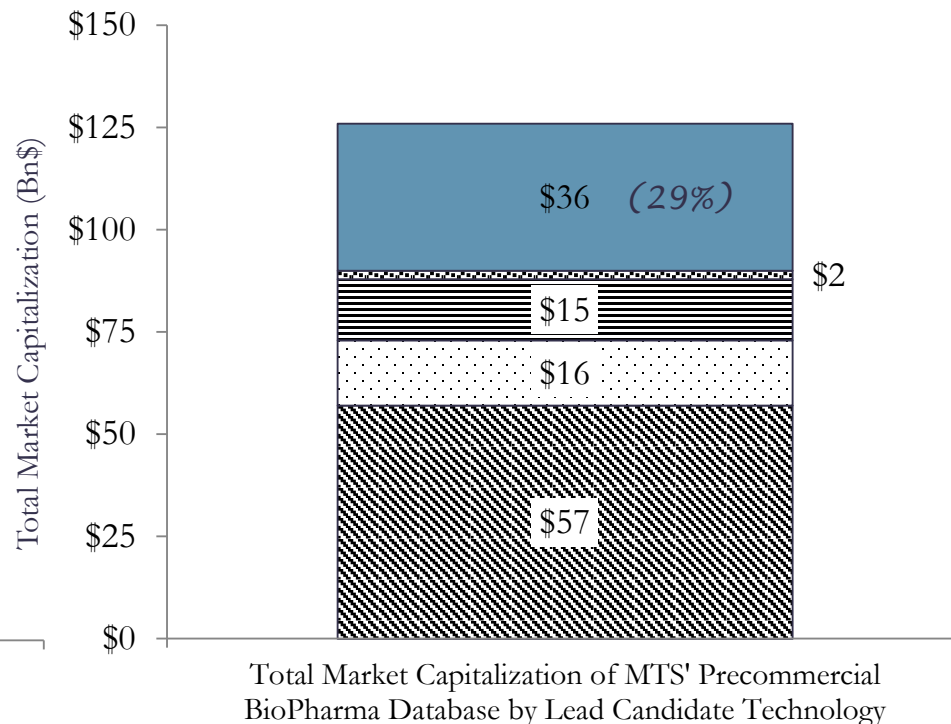
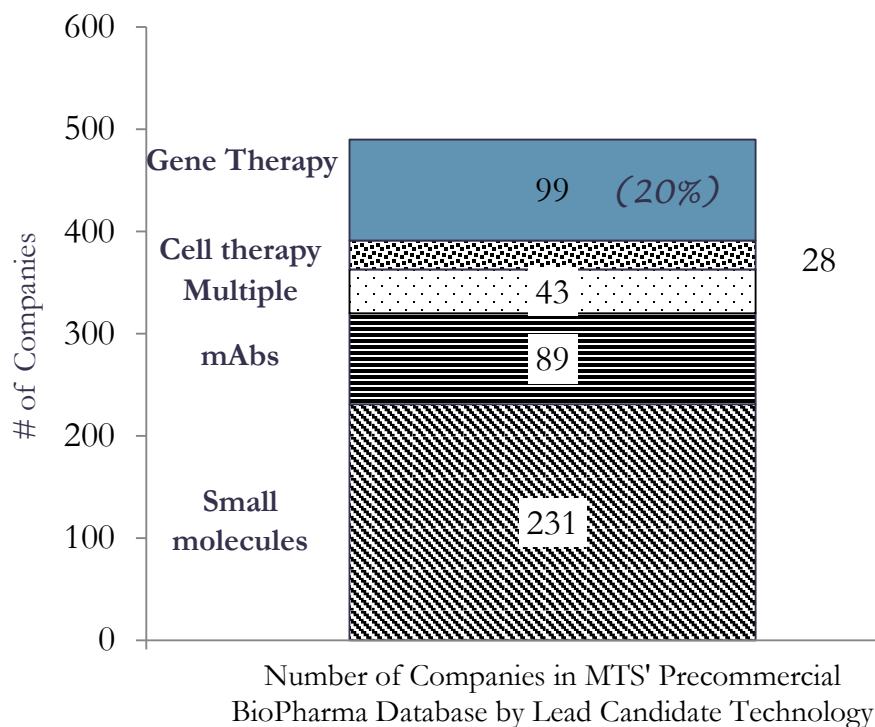


II. Introduction

Intro: Gene Therapy is a Large Component of Precommercial BioPharma*

Intro

20% by number and 29% by market cap – bigger share than mAbs



*MTS' Precommercial BioPharma database includes all companies without a filed, approved, or marketed product

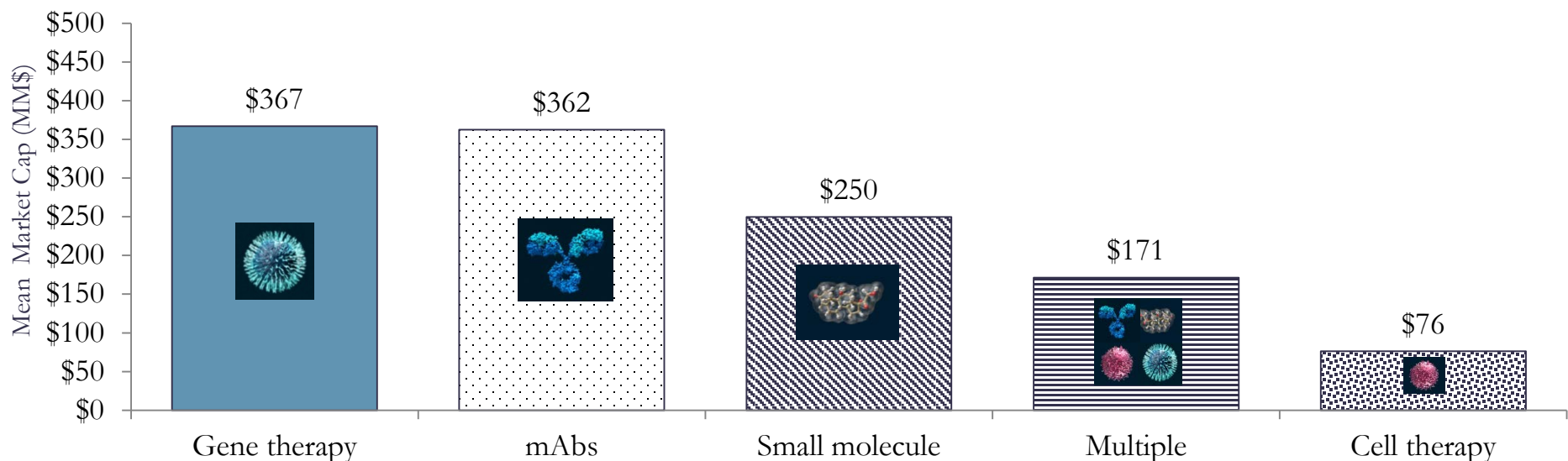
Source: EvaluatePharma, MTS' analysis as of 7/11/2017

Intro: Gene Therapy Valuation Higher than Proven Drug Technologies

Intro

Mean valuation for Gene Therapy companies higher than mAbs and small molecules

Technology of Lead Candidate	# of Companies*	#Companies with MC > \$150 MM	%Companies with MC > \$150 MM
Small molecule	231	75	32%
Gene therapy	99	34	34%
Multiple	89	25	28%
mAb	43	22	51%
Cell therapy	28	4	14%



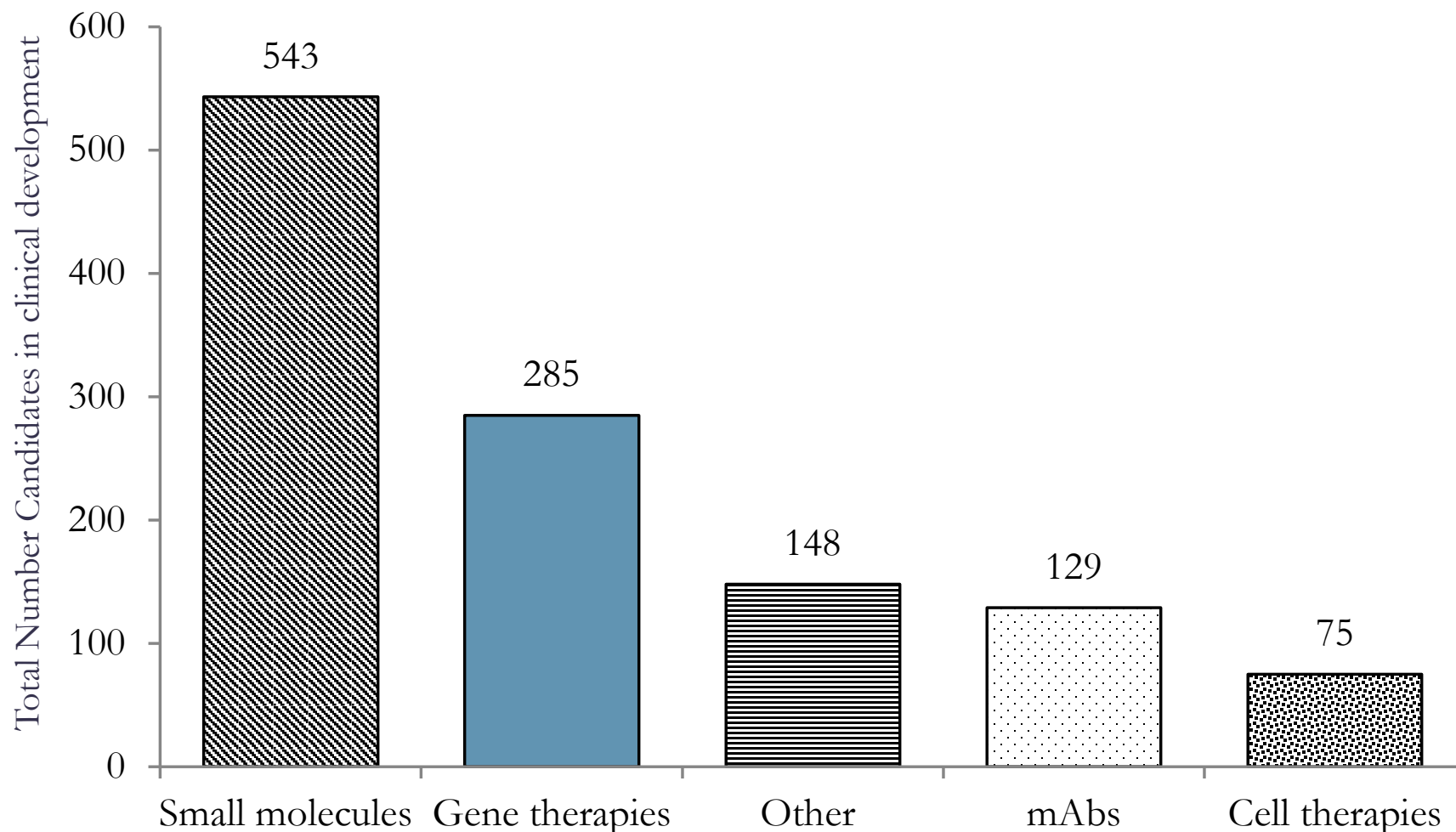
*Categorized based on MTS' analysis of company pipelines

Source: EvaluatePharma; MTS' analysis as of 7/11/17

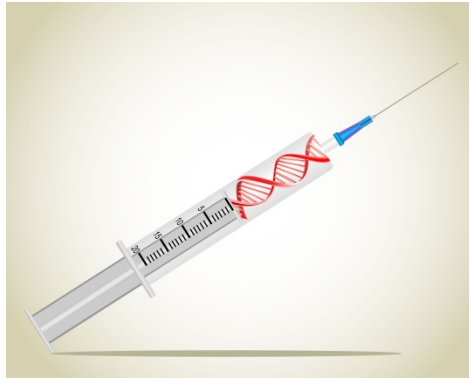
Intro: More than 2X as Many Gene Therapies in Development than mAbs

Intro

Universe of clinical candidates in MTS' precommercial biopharma database



Source: Evaluate Pharma; Equity Research; MTS' analysis as of 7/11/2017

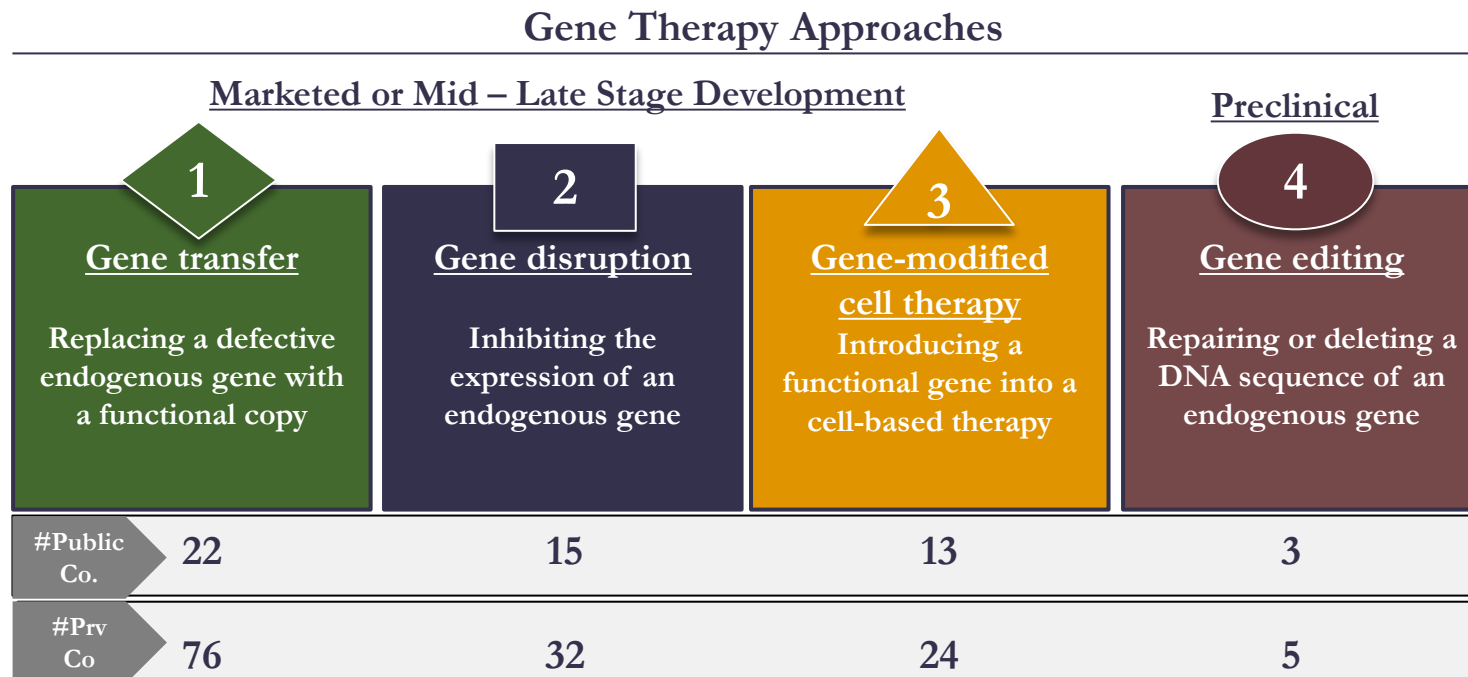


III. Analysis of the Gene Therapy Space

Four Distinct Approaches of Gene Therapy

Analysis

- MTS defines Gene Therapy as the use of genetic material (DNA and RNA) to treat a broad range of diseases, from monogenic disorders to complex disease such as cancer
- Using this definition, we created a proprietary pure-play Gene Therapy database, which include 53 public and 137 private companies
- We have divided companies within our database into one of the following four Gene Therapy approaches based on lead candidate mechanism of action: (1) Gene transfer, (2) Gene disruption, (3) Gene-modified cell therapy, and (4) Gene editing
- In this report, we look at valuation, market potential, BD&L activity and performance through the lens of these four Gene Therapy approaches

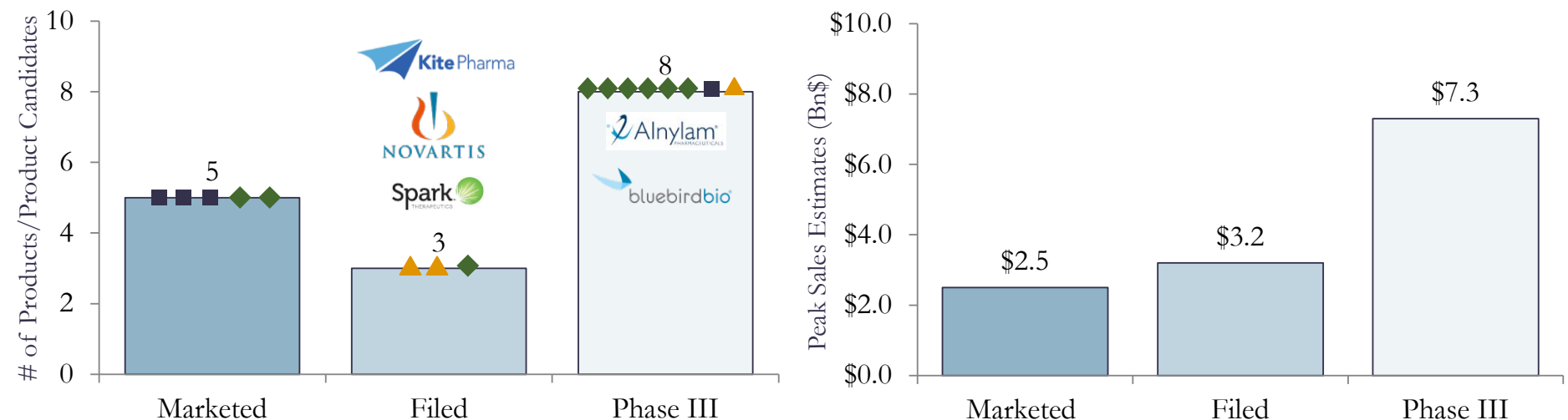


Source: EvaluatePharma, Analysis of MTS' Proprietary Pure-Play Gene Therapy Database as of 7/11/2017

Gene transfer and disruption Approaches have been Approved

Analysis

Approval for 3rd Approach, Gene-modified cell therapy, expected by YE:17



WW Phase	Product / Lead Candidate	Launch date	Company	Market Cap (m\$)	Indication	Approach	Pharmacological Class	Peak Sales (m\$)
Marketed (EU)	Strimvelis	Oct-16	GlaxoSmithKline	\$101,052	SCID	Gene transfer	Adenosine deaminase gene therapy	\$80
Marketed	Spinraza	Dec-16	Biogen	58,441	Musculoskeletal	Gene disruption	Muscular atrophy RNAi therapeutic	2,470
Marketed	Kynamro	Mar-13	Ionis (Kastle)	6,384	Cardiovascular	Gene disruption	Apolipoprotein B-100 antisense	50
Marketed	Exondys 51	Sep-16	Sarepta	1,871	Musculoskeletal	Gene disruption	Exon 51-skipping antisense	1,152
Marketed (EU)	Glybera	Dec-14	uniQure	165	Miscellaneous	Gene transfer	Lipoprotein lipase gene therapy	-
Filed	CTL019	2018	Novartis	\$193,766	Cancer	Gene-modified cell therapy	Anti-CD19 CAR T cell therapy	\$1,212
Filed	Axicabtagene ciloleucel	2018	Kite	5,718	Cancer	Gene-modified cell therapy	Anti-CD19 CAR T cell therapy	1,592
Filed	Voretigene neparvovec	2018	Spark	1,864	Sensory organs	Gene transfer	RPE gene therapy	371

Phase III	Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)
Phase III	
Phase III	
Phase III	
Phase II/III	
Phase III	
Phase III	
Phase III	

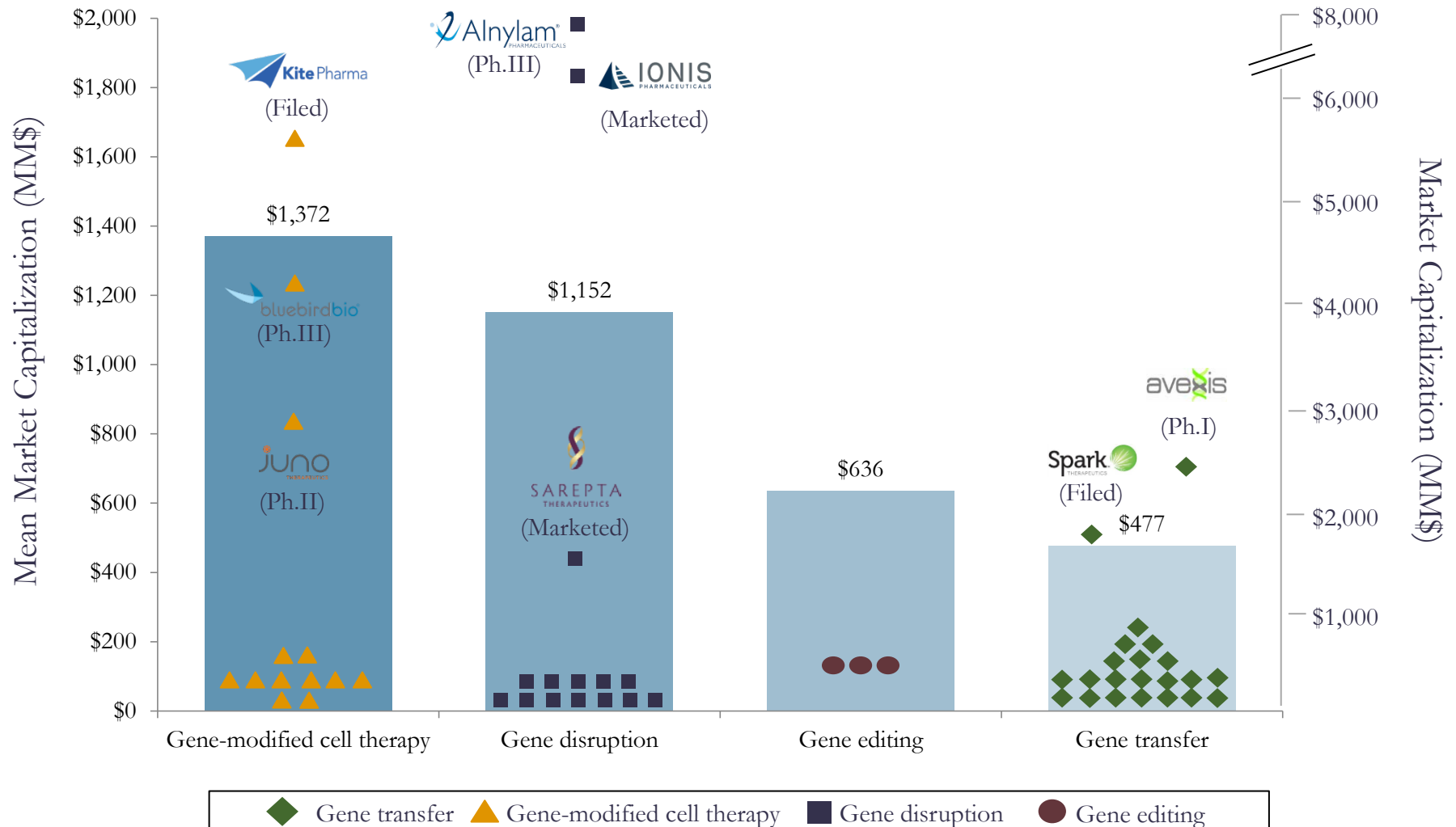
◆ Gene transfer
▲ Gene-modified cell therapy
■ Gene disruption
● Gene editing

Source: EvaluatePharma, Equity Research, MTS' analysis as of 7/11/2017

Valuations Differ Within & Across Gene Therapy Approaches

Analysis

Larger market caps for companies with potential near-term product approvals

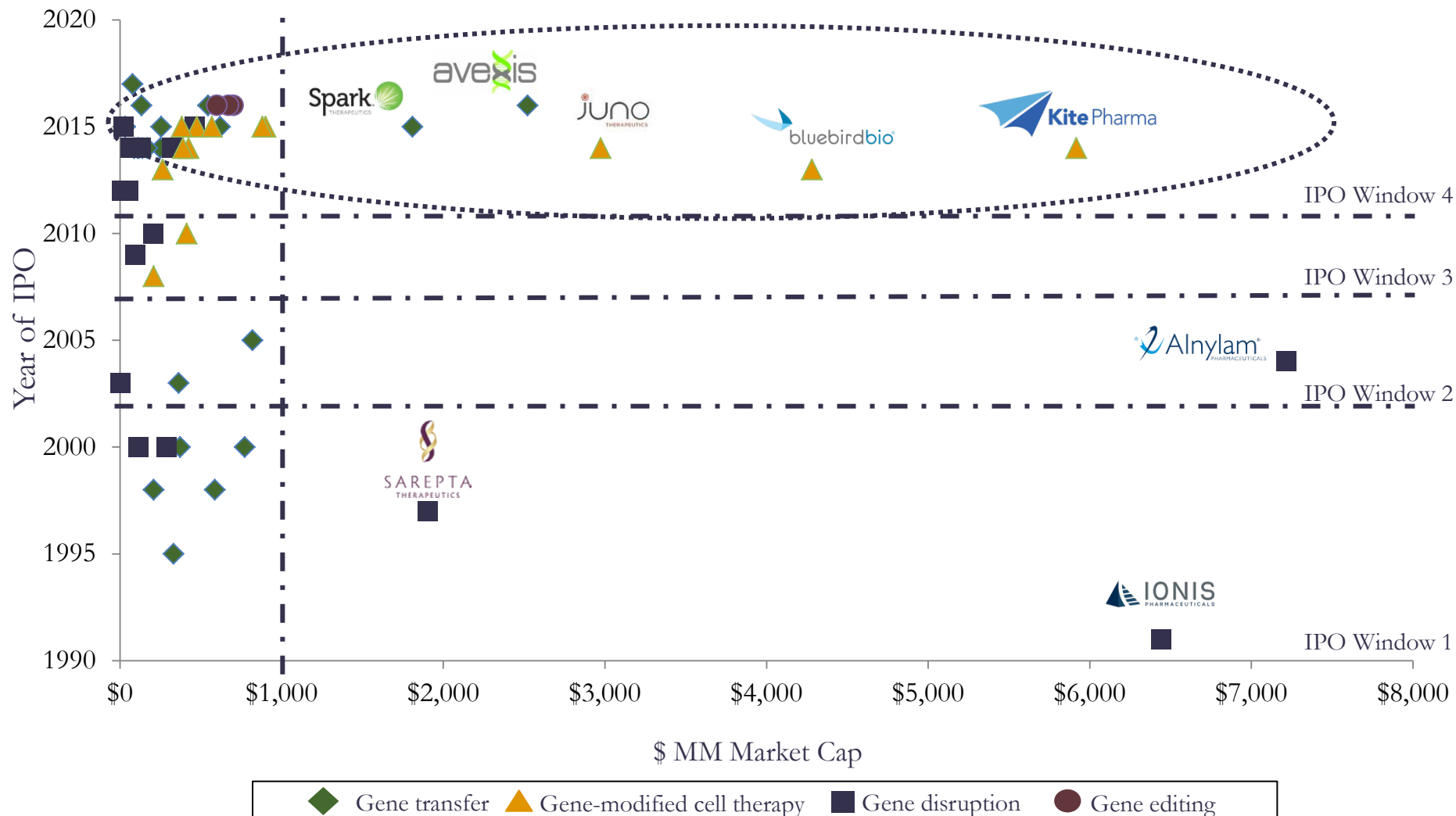


Source: CapIQ, Analysis of MTS' Proprietary Pure-Play Gene Therapy Database as of 7/11/2017

Senior Companies are not Necessarily Valuation Leaders

Analysis

Recent IPO window has produced the majority of companies with largest valuations

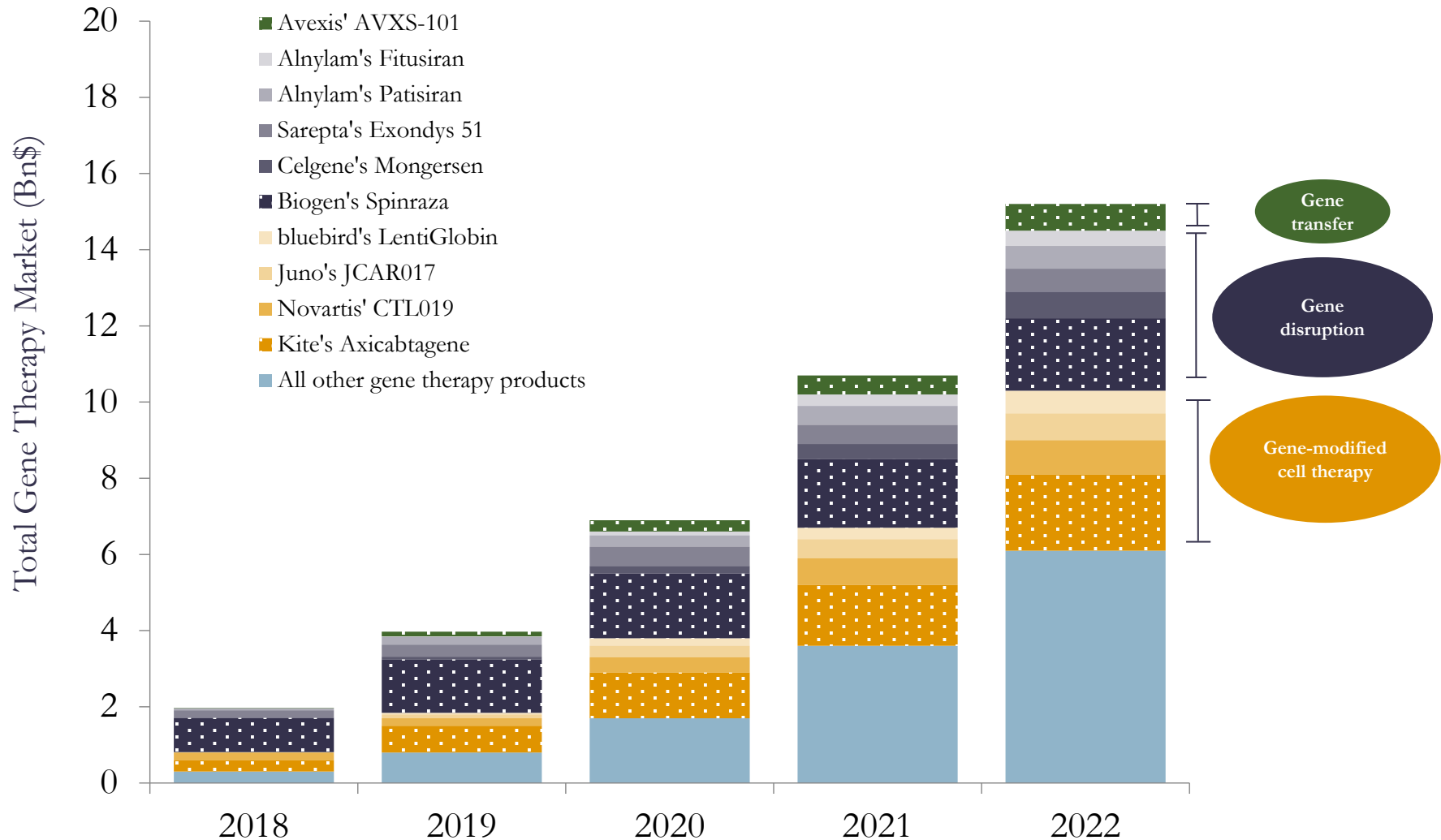


Source: CapIQ, Analysis of MTS' Proprietary Pure-Play Gene Therapy Database as of 7/11/2017

Two Gene Therapy Approaches to Drive Market Growth

Analysis

Gene disruption and Gene-modified cell therapies expected to grow market 8X by 2022

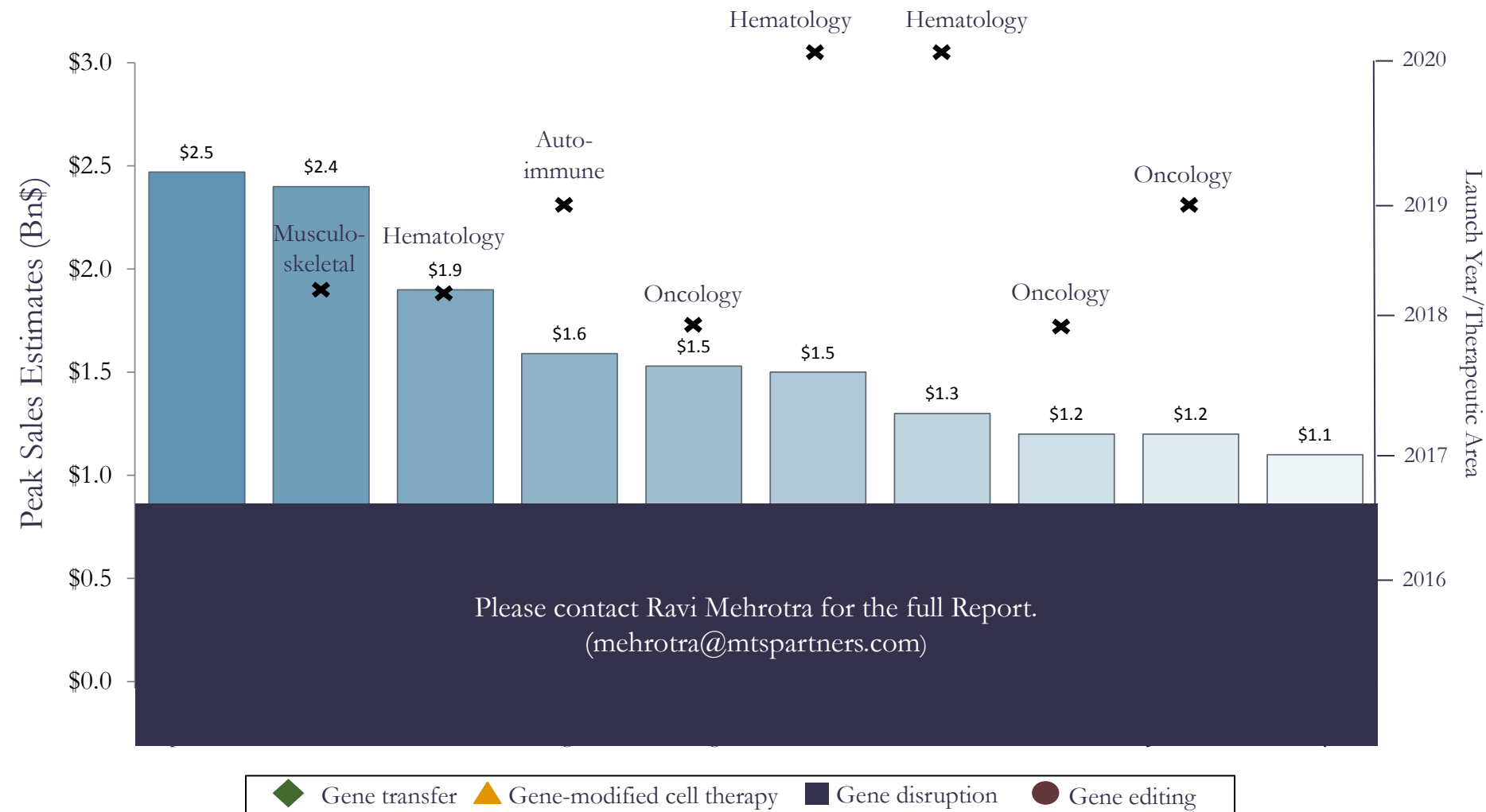


Source: Evaluate Pharma; Equity Research, MTS' analysis as of 7/11/2017

Blockbusters Expected in at Least Three Gene Therapy Approaches

Analysis

Peak sales estimates for marketed as well as mid/late-stage candidates







































Source: Bloomberg consensus estimates, Equity Research, EvaluatePharma, MTS' analysis as of 7/11/2017

Modest M&A Appetite for Gene Therapy Approaches

Acquisitions mainly for Preclinical/Ph.I companies with platform technologies

Analysis

Development Stage of Acquired Assets	# of Acquisitions	Acquired Companies	Acquirers
Approved / Phase III	1		
Phase II	6	     	     
Phase I/ Preclinical	11	          	          

Source: Evaluate Pharma, Equity Research, MTS' analysis as of 7/11/2017

M&A Activity Equal Across All Gene Therapy Approaches

Analysis

Details of select M&A deals since 2013

Transaction Date	Company	Acquirer	Upfront Payment (m\$)	Deal Value (m\$)	Class	Lead Product / Program	Status on Trans. Date	Transaction Comment
Jan-17								
Sep-16								
Aug-16								
May-16								
Mar-16								
Mar-16								
Sep-15								
May-15								
Feb-15								
Jan-15								
Sep-14								
Aug-14								
Jun-14								
Mar-14								
Jan-14								
Oct-13								
Oct-13								
Jul-13								














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Source: Evaluate Pharma, Equity Research, MTS' analysis as of 7/11/2017

Preference to Partner Early-stage Assets/Platform Technologies

Analysis

Licensing deals mainly for Preclinical/Ph.I candidates

Development Stage of Licensed Assets	# of Licensing Deals	Licensor	Licensee
Marketed / Phase III	2	 	 
Phase II	5	   	    
Phase I/ Preclinical	31	<div>Adicet Bio</div> <div>CRT Pioneer</div> <div>Idera</div> <div>MD Anderson</div> <div>uniQure</div> <div>Arrowhead</div> <div>Dimension</div> <div>Intellia</div> <div>Moderna</div> <div>Viromed</div> <div>BioNTech</div> <div>Editas</div> <div>Intrexon</div> <div>Nitto Denko</div> <div>WAVE</div> <div>Celyad</div> <div>Exicure</div> <div>Ionis</div> <div>Precision Bio</div> <div>CRISPR</div> <div>Five Prime</div> <div>Kite</div> <div>Spark</div>	<div>Allergan</div> <div>Juno</div> <div>Amgen</div> <div>Merck</div> <div>AstraZeneca</div> <div>Merck KGaA</div> <div>Bayer</div> <div>Ono</div> <div>bluebird bio</div> <div>Pfizer</div> <div>Bristol-Myers</div> <div>Regeneron</div> <div>Fosun</div> <div>Sanofi</div> <div>GlaxoSmithKline</div> <div>Shire</div> <div>Vertex</div> <div>Intrexon</div> <div>JNJ</div>

Source: Evaluate Pharma, Equity Research, MTS' analysis as of 7/11/2017

Licensing Activity Equal Across All Gene Therapy Approaches

Analysis

Details of select licensing deals since 2014

Deal Date	Licensor	Licensee	Geography	Upfront Payment (\$m)	Deal Value (\$m)	Class	Candidate/Program	Indication	Status on Deal Date
May-17									
Mar-17									
Feb-17									
Jan-17									
Jan-17									
Jan-17									
Jan-17									
Dec-16									
Nov-16									
Sep-16									
Sep-16									
Aug-16									
Jul-16									
Jul-16									
Jun-16									
May-16									
May-16									
Apr-16									
Feb-16									
Jan-16									
Dec-15									
Nov-15									
Nov-15									
Oct-15									
Aug-15									
Jul-15									
Jun-15									
May-15									
May-15									
May-15									
Apr-15									
Mar-15									
Feb-15									
Jan-15									
Jan-15									
Jan-15									
Dec-14									
Jun-14									

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Source: Evaluate Pharma, Equity Research, MTS' analysis as of 7/11/2017

Large Cap Showing Slight Preference for Gene transfer Approach

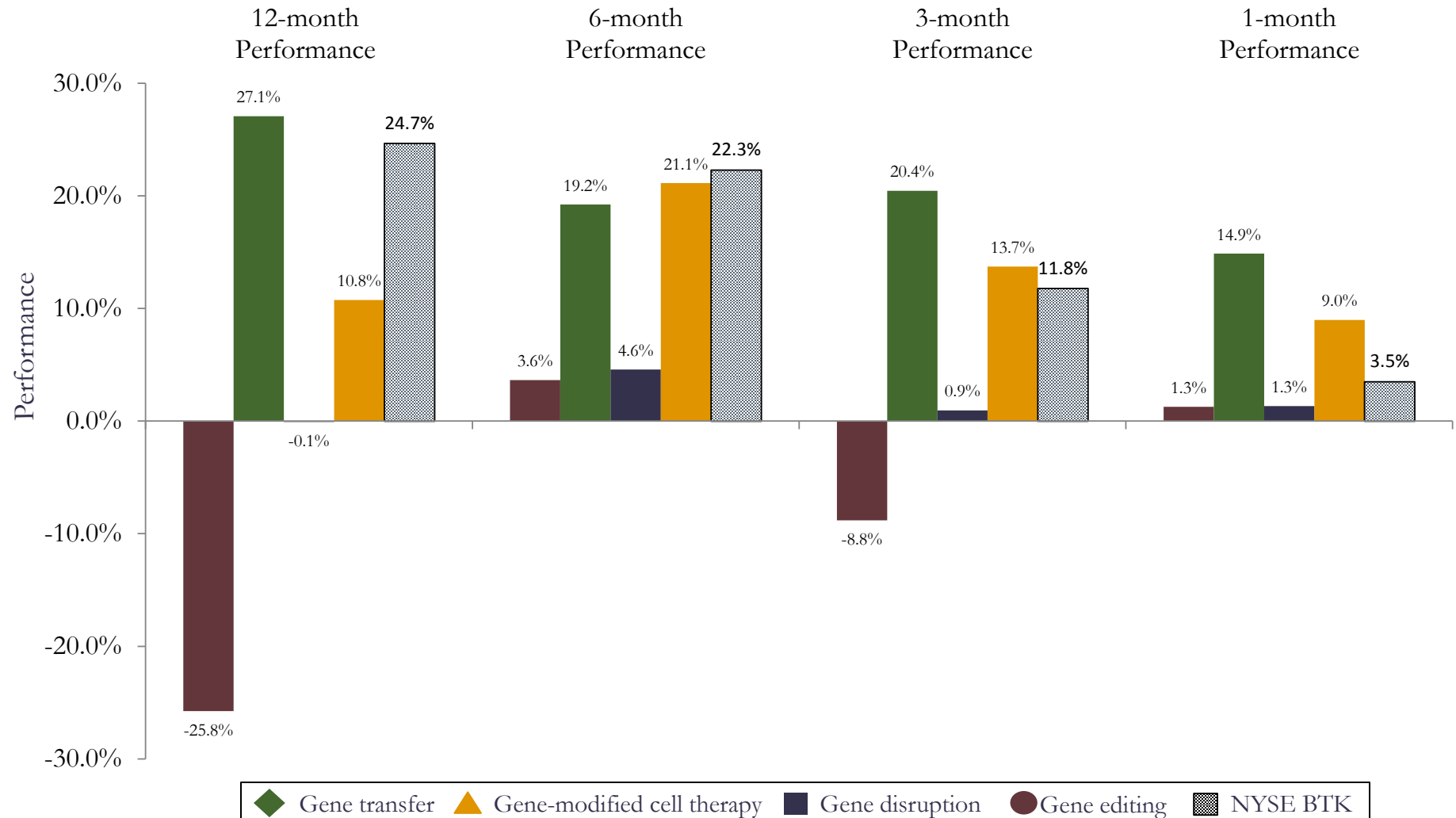
Analysis

Analysis of top 10 Gene Therapy licensees since 2014



Aggregate Market Performance for Companies within each Gene Therapy approach

Analysis



Source: Source: CapiQ, Analysis of MTS' Proprietary Pure-Play Gene Therapy Database as of 7/11/2017

Select Near-term Catalysts for Select Gene Therapy Companies

Analysis

Date	Company	Ticker	Events
Mid-2017	◆		
Mid-2017	◆		
Mid-2017	◆		
Mid-2017	◆		
Mid-2017	■		
Mid-2017	■		
Mid-2017	■		
Mid-2017	◆		
Mid-2017	■		
3Q'17	◆		
3Q'17	◆		
3Q'17	■		
3Q'17	◆		
3Q'17	◆		
3Q'17	◆		
3Q'17	◆		
3Q'17	◆		
3Q'17	■		
Late 3Q'17	■		
2H'17	▲		
2H'17	◆		
2H'17	▲		
2H'17	▲		
4Q'17	▲		
YE'17	▲		
YE'17	●		
YE'17	●		
YE'17	■		
YE'17	▲		
YE'17/1Q'18	■		

Please contact Ravi Mehrotra for the full Report.
(mehrotra@mtspartners.com)

Source: Equity Research, Biomed Tracker, MTS' analysis as of 7/11/2017

Near term Catalysts for Select Gene Therapy Companies

Analysis

Date	Company	Ticker	Events
YE'17/1Q'18			Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)
1Q'18			
1Q'18			
1H'18			
Mid-2018			

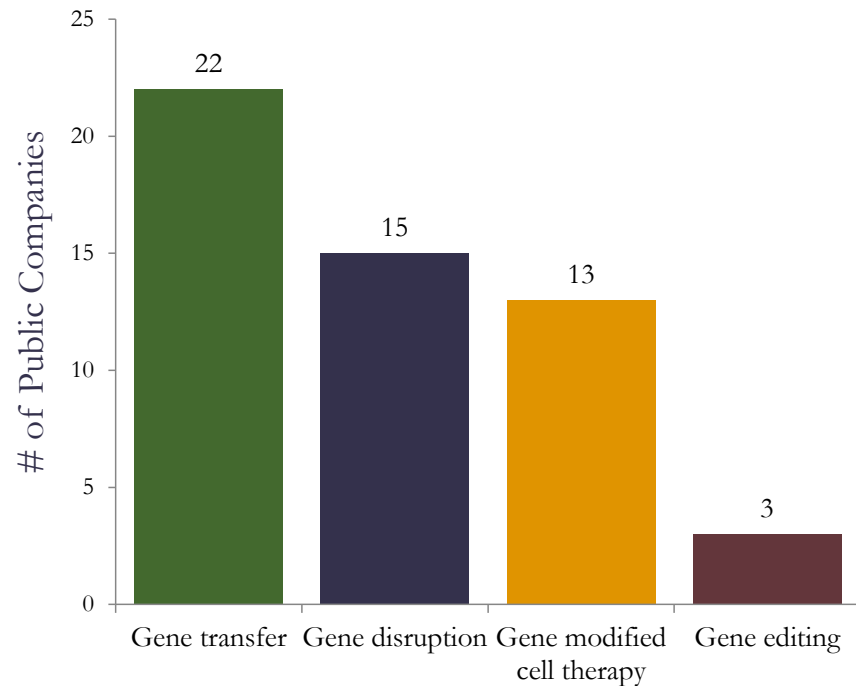


IV: Appendix

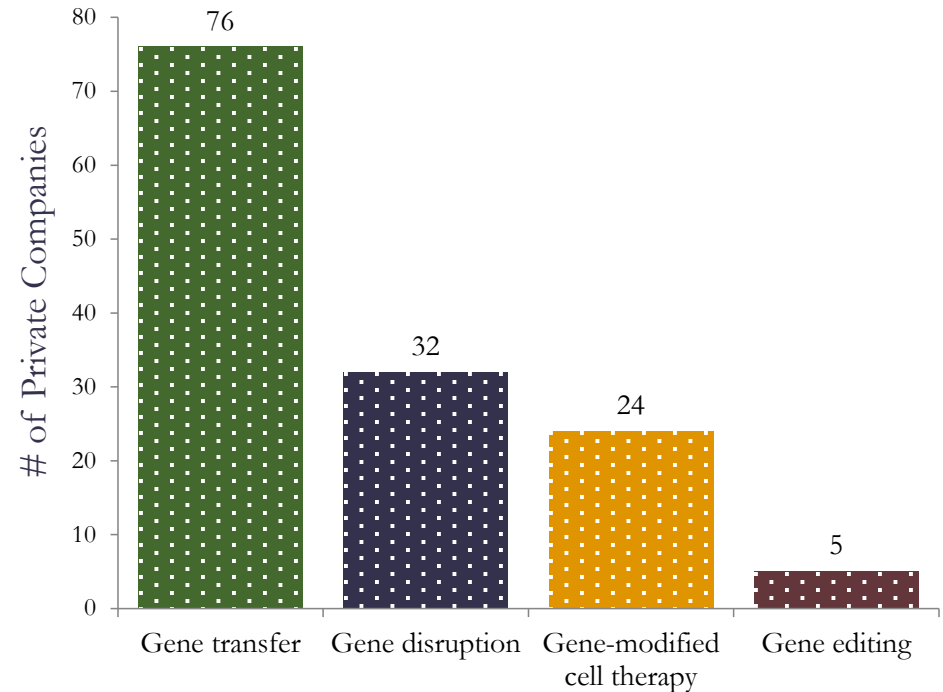
Gene Therapy Approaches in MTS' Public and Private Sector Pure-Play Gene Therapy Database*

Appendix

Public Companies



Private Companies



Footnote: *Companies included in MTS' Public and Private Gene Therapy Database are listed on Appendix slides #XX

Source: EvaluatePharma, MTS analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Sorted on Market Capitalization

Appendix

Company	Market Cap (m\$)	Approach	Product/ Lead Candidate	WW Phase	Indication	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)						

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Sorted on Market Capitalization

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Company	Market Cap (m\$)	Approach	Product/ Lead Candidate	WW Phase	Indication	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)						

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Private Pure-Play Gene Therapy Data

Alphabetical order

Appendix

Company	Approach	Lead Candidate	WW Phase	Indication	Pharmacological Class
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Please contact Ravi Mehrotra for the full Report.
(mehrotra@mtspartners.com)

MTS' Proprietary Private Pure-Play Gene Therapy Data

Alphabetical order

Appendix

Company	Approach	Lead Candidate	WW Phase	Indication	Pharmacological Class
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Please contact Ravi Mehrotra for the full Report.
(mehrotra@mtspartners.com)

MTS' Proprietary Private Pure-Play Gene Therapy Data

Alphabetical order

Appendix

Company	Approach	Lead Candidate	WW Phase	Indication	Pharmacological Class
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(mehrotra@mtspartners.com)

MTS' Proprietary Private Pure-Play Gene Therapy Data

Alphabetical order

Appendix

Company	Approach	Lead Candidate	WW Phase	Indication	Pharmacological Class
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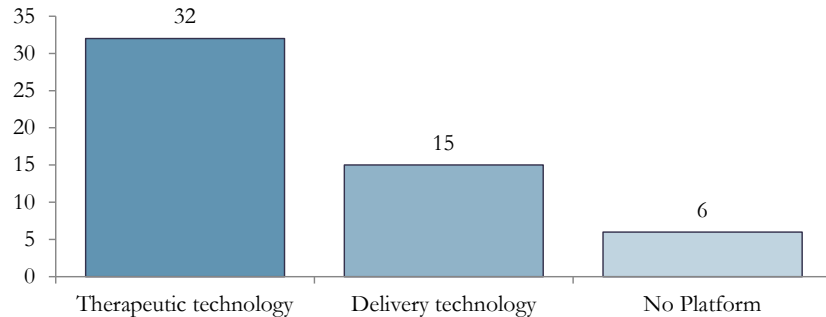
Please contact Ravi Mehrotra for the full Report.
(mehrotra@mtspartners.com)

Features of Public Gene Therapy Companies

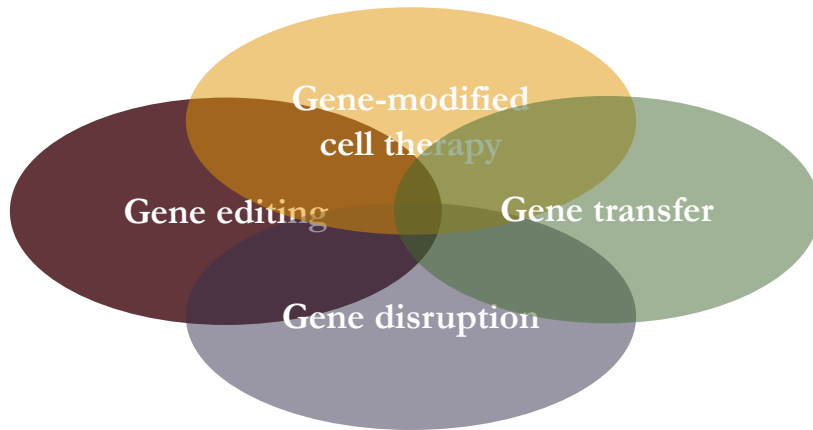
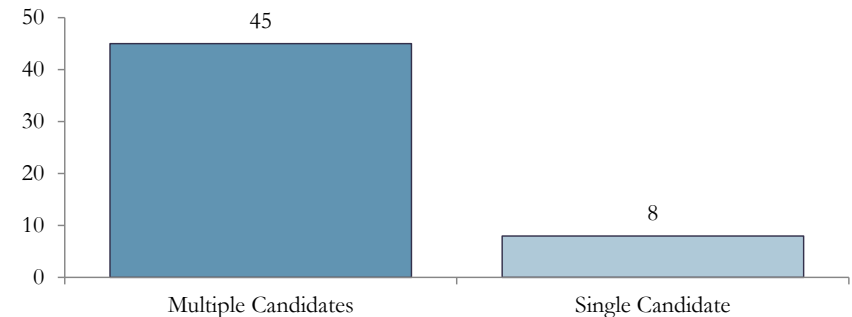
Appendix

Platforms and multi-asset pipelines dominate across Gene Therapy approaches

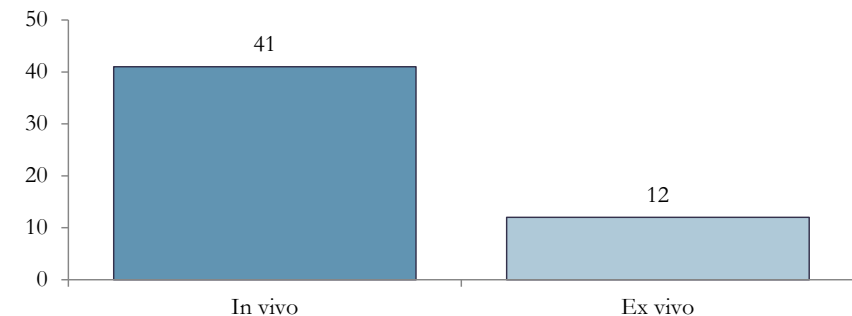
Most Companies Developing a Specialized Platform*



Most Companies Developing Multiple Drug Candidates



Most Companies Developing In vivo Therapies**



Footnote: *Therapeutic platform – examples include CARs or special chemistries for RNA interference (RNAi); Delivery platform – examples include specialized vectors for Gene Therapy delivery to specific cell types. **In vivo – Gene Therapy candidate is administer directly to the patient; Ex vivo – Patient’s cells are removed from the body, Gene Therapy is applied, and cells are reintroduced into the body

Source: MTS’ analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Sorted on platform

Appendix

Platform	Company	Market Cap (m\$)	In vivo or Ex vivo	Lead Candidate	Indication	WW Phase	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Sorted on platform

Appendix

Platform	Company	Market Cap (m\$)	In vivo or Ex vivo	Lead Candidate	Indication	WW Phase	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Appendix

Sorted on *in vivo* or *ex vivo* therapy

In vivo or Ex vivo	Company	Ticker	Market Cap (m\$)	Product / Lead Candidate	WW Phase	Indication	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

Appendix

Sorted on *in vivo* or *ex vivo* therapy

In vivo or Ex vivo	Company	Ticker	Market Cap (m\$)	Product / Lead Candidate	WW Phase	Indication	Pharmacological Class
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

2022 sales estimates for select Gene-modified cell therapies



Appendix

Product	WW Phase	Company	Indication	Pharmacological Class	2022 sales (MM\$)
---------	----------	---------	------------	-----------------------	----------------------

Please contact Ravi Mehrotra for the full Report.
(mehrotra@mtspartners.com)

MTS' Proprietary Public Pure-Play Gene Therapy Data

2022 sales estimates for select Gene disruption therapies



Appendix

Product	WW Phase	Company	Indication	Pharmacological Class	2022 sales (MM\$)
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Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)					
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Source: EvaluatePharma, MTS' analysis as of 7/11/2017

MTS' Proprietary Public Pure-Play Gene Therapy Data

2022 sales estimates for select Gene transfer therapies

Appendix

Product	WW Phase	Company	Indication	Pharmacological Class	2022 sales (MM\$)
---------	----------	---------	------------	-----------------------	----------------------

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(mehrotra@mtspartners.com)

MTS' Proprietary Public Precommercial Database

Sorted by lead candidate therapeutic technology

Appendix

Technology	Company	Symbol	Market Cap (MM\$)	Lead Candidate	WW Phase	Therapeutic Area	Indication
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis of company pipelines

MTS' Proprietary Public Precommercial Database

Sorted by lead candidate therapeutic technology

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Gene therapy	Genevec	GNVC	0	TRILZATOP	Phase II	Sensory	Other ear disorders

Source: EvaluatePharma, MTS' analysis of company pipelines

MTS' Proprietary Public Precommercial Database

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Source: EvaluatePharma, MTS' analysis of company pipelines

MTS' Proprietary Public Precommercial Database

Sorted by lead candidate therapeutic technology

Appendix

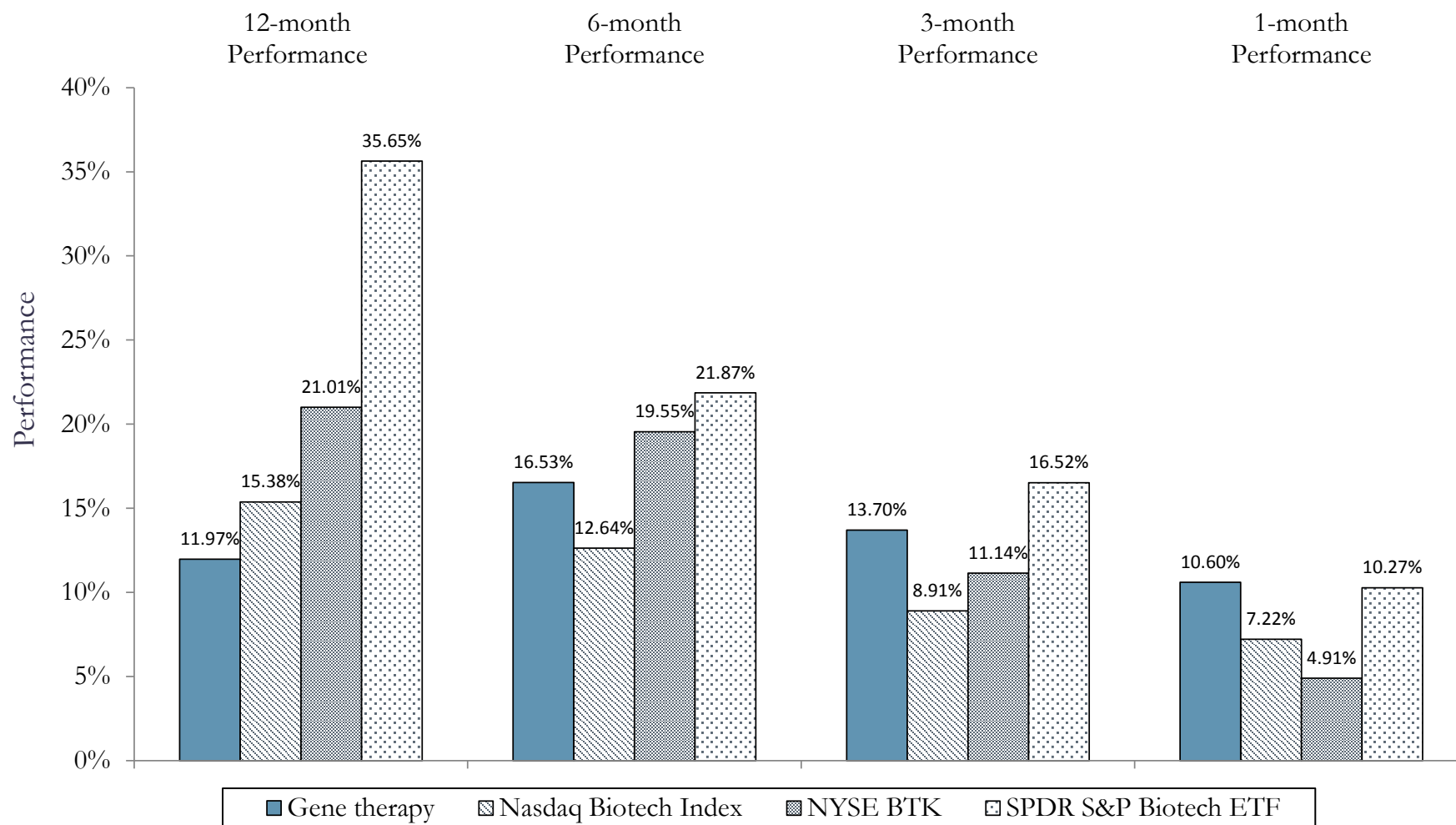
Technology	Company	Symbol	Market Cap (MM\$)	Lead Candidate	WW Phase	Therapeutic Area	Indication
Please contact Ravi Mehrotra for the full Report. (mehrotra@mtspartners.com)							

Source: EvaluatePharma, MTS' analysis of company pipelines

Gene Therapy Sector Performance YTD In Line with Broader Biotech

Appendix

Gene Therapy has had a strong 1H:17



Source: CapIQ, Analysis of MTS' Proprietary Pure-Play Gene Therapy Database as of 7/11/2017

MTS' Scale is Comparable to Healthcare Groups of Major Banks

But with the Focused Attention of a Boutique

Broad Franchise

**MTS Founded by
Curtis Lane in 2000**

*Started and Ran Bear Stearns'
Healthcare Banking from 1983 - 1999*

11 Partners

**8 Directors / Vice
Presidents**

**25 Analysts /
Associates**

Dedicated Strategic Advisory Analytics Team*

Ravi Mehrotra, Ph.D.

Partner

- Previously, Ravi was an equity research analyst at Credit Suisse for 11 years and most recently held the title of Global Head of Biotechnology Equity Research
- Prior to joining MTS in 2016, Ravi was the Chief Corporate Development Officer at OvaScience

Anamaria Sudarov, Ph.D.

Vice President

- Prior to joining MTS in 2016, Dr. Sudarov was a Senior Associate within Major Pharma/Life Sciences team at Credit Suisse
- Prior to that she worked on Global Biotech Equity Research team covering both Large Cap and Small/Mid Cap Biotech stocks at Credit Suisse

Ryan Cinalli, Ph.D.

Associate

- Prior to joining MTS in 2016, Ryan was an equity research analyst at LifeSci Capital, where he covered Small Cap Biotechnology companies

Senior Life Sciences Team*

Peter Collum

Partner

- Has worked on over \$20 billion in M&A deal volume
- Director, Bank of America Healthcare Investment Banking from 2003-2009
- Prior to BofA, Technical Development Engineer at Hoffmann-La Roche

Mark Epstein

Partner

- Completed over 100 private financing for clients raising over \$5 billion in private capital
- Managing Director / Co-Head Bank of America Private Equity Placements
- Prior to BofA, co-head of Direct Private Equity Placements at Merrill Lynch

**Andrew
Fineberg**

Partner

- Prior to MTS, Andrew led the Financing Group at Torrey Partners, LLC, a boutique investment bank
- Prior to Torrey Partners, Andrew worked at Cowen and Company, a global investment bank as Vice President in the Private Placement Group

David Low

Partner

- Joined MTS as a Partner in 2017
- Previously, Managing Director since 2002 in the Life Sciences Group at Lazard
- Prior to Lazard, David was a Managing Director at JPMorgan

**Andrew
Weisenfeld**

Partner

- Has worked on approximately \$50 billion in M&A deal volume across a range of advisory transactions
- Managing Director/ Co-Head of BofA Life Sciences Investment Banking
- Prior to BofA, Managing Director and Head of Healthcare M&A at JPMorgan

Soojin Kwon

Partner

- Has worked on over \$14 billion in equity and M&A deal volume
- Director, Bank of America Healthcare Equity Capital Markets
- Prior to BofA, Equity Research Analyst at Merkin Biomed Asset Management and UBS, respectively
- Also, formerly worked in pharma consulting

*Team members that are partners are limited partners of MTS Health Partners, L.P. as well as registered representatives of MTS Securities, LLC, an SEC registered securities broker-dealer and FINRA member