



NYSE American: PFNX

# Corporate Presentation

May 2019

**PFE***nex*

*Innovative Solutions for Global Health*

# Pfenex

## Safe Harbor Statement

This presentation, including the accompanying oral presentation (the “Presentation”), includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, which are based on current expectations, estimates and projections based on information currently available to management. These forward-looking statements include, among others, statements regarding the future potential of Pfenex’s product candidates, including future plans to advance, develop, manufacture and commercialize its product candidates, including the expected commercial strategy for PF708 depending on type of FDA approval; potential market opportunities for Pfenex’s product candidates including, PF708, PF582, and PF529; the potential FDA approval of the NDA for PF708 and the earliest potential commercial US launch of PF708 in the fourth quarter of 2019; Pfenex’s 2019 focus and strategy; the expected patent expiration timelines and strategies for Forteo and other branded reference drugs; developments and projections relating to competitors and the industry, including that, if launched, there may be limited competition for PF708; expectations with regard to future milestone, royalty, and other payments from Pfenex’s collaborations with Jazz Pharmaceuticals, Alvogen, NT Pharma, Merck, SII and other third parties; Pfenex’s expectations with respect to the advancement of PF743 and PF745 with Jazz Pharmaceuticals; Pfenex’s expectations regarding regulatory submissions; Pfenex’s expectations regarding the use of abbreviated regulatory pathways for the approval of its product candidates, including use of the 505(b)(2) regulatory pathway for PF708 and the 351(k) pathway for PF529; Pfenex’s expectations regarding the timing and advancement of clinical trials and the types of future clinical trials for its product candidates; Pfenex’s expectations regarding its well defined IP strategy; and Pfenex’s expectation for potential strategic partnership opportunities to maximize value for advancement of PF582, PF529, and its other product candidates. Forward-looking statements are typically identified by words like “believe,” “anticipate,” “could,” “should,” “estimate,” “expect,” “intend,” “plan,” “project,” “will,” “forecast,” “budget,” “pro forma,” and similar terms. Factors that could cause Pfenex’s results and expectations to differ materially from those expressed in forward-looking statements include, without limitation, Pfenex’s need for additional funds to support its operations; its success being dependent on PF708; Pfenex’s reliance on its collaboration partners’ performance over which Pfenex does not have control; failure to achieve favorable results in clinical trials its product candidates or receive regulatory approval; delays in its clinical trials or in enrollment of patients in its clinical trials; failure to market PF708, or its other product candidates due to the existence of intellectual property protection owned or controlled by a third party and directed to PF708, or its other product candidates; PF708, and its other product candidates may cause serious adverse side effects or have properties that delay or prevent regulatory approval or limit their commercial profile; if approved, risks associated with market acceptance, including pricing and reimbursement; Pfenex’s ability to enforce its intellectual property rights; adverse market conditions; and changes to laws and government regulations involving the labelling, approval process, funding and other matters affecting biosimilars, therapeutic equivalents to branded products and vaccines. Pfenex has not received marketing approval for any product candidates, nor has Pfenex launched any products, and there is no certainty that any marketing approvals will be obtained, products launched, or as to the timelines on which they will occur. Further, even if Pfenex obtains marketing approval, Pfenex may be subject to direct legal challenges by the manufacturers of reference products and Pfenex could be delayed or prevented from launching its product candidates, including PF708, as a result of court orders or as a result of the time necessary to resolve such challenges. Unless otherwise indicated, forward-looking statements represent Pfenex’s management’s beliefs and assumptions only as of its May 9, 2019 press release announcing results for the quarter ended March 31, 2019. You should read Pfenex’s Quarterly Report on Form 10-Q for the quarter ended March 31, 2019, and Pfenex’s Annual Report on Form 10-K for the year ended December 31, 2018, including the Risk Factors set forth therein, and its subsequent reports filed with the SEC, including the Risk Factors set forth therein, completely and with the understanding that Pfenex’s actual future results may be materially different from what Pfenex expects. Except as required by law, Pfenex assumes no obligation to update these forward-looking statements publicly, or to update the reasons why actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.

# Pfenex

## Corporate overview

**Pfenex is a clinical stage development and licensing biotechnology company focused on leveraging its Pfenex Expression Technology® platform to develop and improve protein therapies for unmet patient needs**



### Validated Platform

Clinically validated platform with our products and our partners' products nearing commercialization



### Differentiated Portfolio

Advanced pipeline of therapeutic equivalents, biologics, vaccines and biosimilars



### Distinct Value Drivers

- PF708, Jazz Partnership, and CRM197
- Up to \$280M+ in upfronts and potential milestones
  - Sales and royalties as early as Q4'19, subject to FDA approvals and other factors



### Anticipated Near-Term Milestones

- PF708 commercial U.S. launch subject to FDA approval and other factors
- Potential Jazz partnership advancements
- Potential CRM197 sales and royalties



### Cash and Shares Outstanding

Cash position of \$45.8M and 31.5M shares outstanding (as of 3/31/2019)

## 2019 key program focus and total program economic opportunities

### 2019 key program focus

### Potential total milestones & royalties

1

**PF708**  
**Teriparatide**

- PDUFA date Oct. 7, 2019 and US launch readiness
- Successful expiration of 45 day waiting period under Hatch-Waxman
- Potential ex-US regulatory submissions

- US: \$27.5M & up to 50% GP
- ROW: \$2.5M & up to 60% GP
- Asia: \$25M & royalties

2

**PF743/PF745**  
**Jazz**

- PF743 on-going development - Jazz responsibility
- Pfenex PF745 advancement per mutually agreed development plans with Jazz

- \$224.5M total MS, \$189M remaining
- \$30M/\$34M, development/regulatory
- \$125M sales
- Tiered royalties on sales

3

**CRM197**

- SII Pneumosil® positive P3 data results, WHO prequalification process
- Clinical progress Merck V114
- Expand CRM197 use in partnered programs

- SII: tiered royalties on net sales
- Merck: annual fees, milestones and tiered royalties on net sales

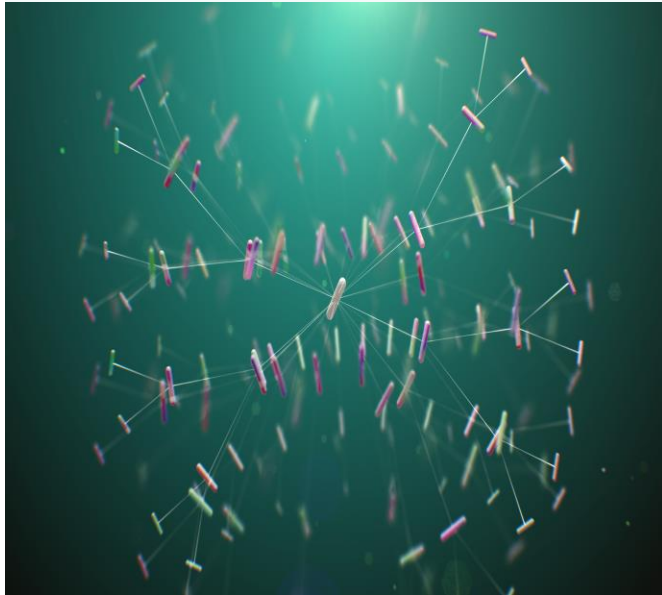
GP = Gross Profit (Net revenue – Cogs)

MS = Milestones

# Pfenex Expression Technology® Platform

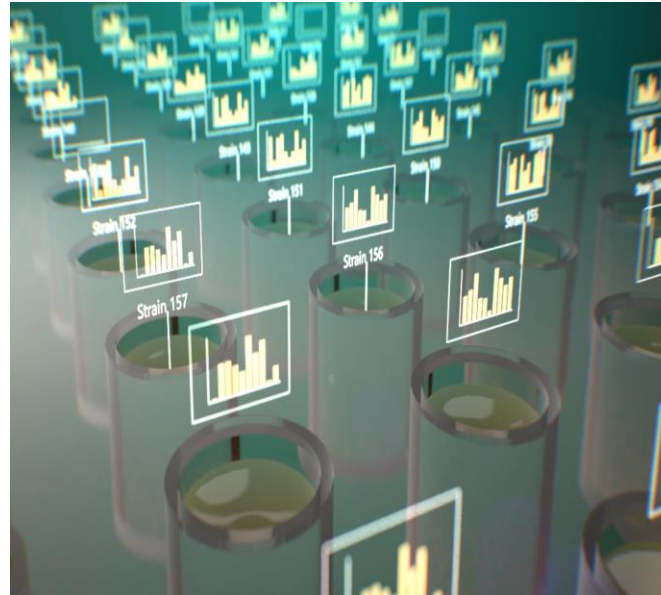
## Parallel, high throughput microbial strain development enables expedited discovery and development

### 1 Engineer 1,000's of Strains



Pfenex leveraged bioinformatics and transcriptomics to develop extensive toolbox of expression strains and plasmids

### 2 Rapid Screening



High throughput screening enables production strains to be screened for titer, solubility, and activity prior to scale-up

### 3 Scale-Up and Production



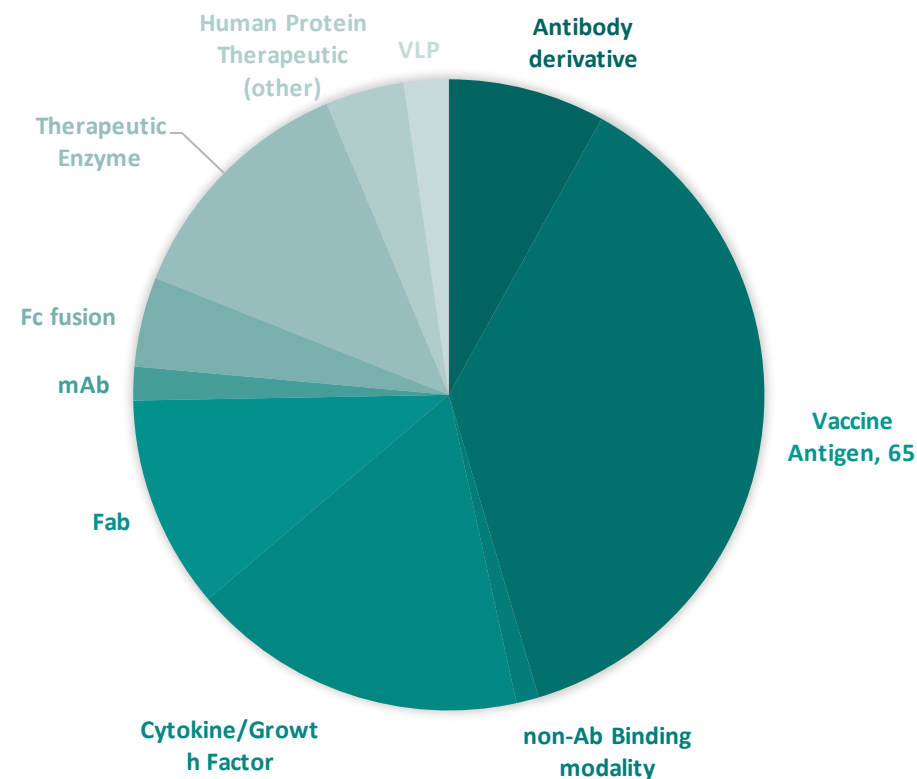
Targeted process optimization and state of the art analytical capabilities deliver commercial manufacturing strain

## Proprietary Platform

# Our platform is uniquely advantaged for next generation modalities

- ▶ Deep institutional knowledge derived from 60+ collaborations including most of top 15 big pharma over 15 years sustaining a success rate\* >80% expressing 174 “lead” proteins.
- ▶ Success rates of 90-100% for Fab’s , engineered antibody derivatives, non-antibody binding modalities and vaccine antigens
- ▶ Broad platform and product IP portfolio with 24 issued U.S. patents as of May 2019.
- ▶ Proprietary platform and seasoned team enable development of novel therapeutics leveraging broad range of next-generation modalities.

Distribution of 174 “lead” protein classes



\*Success is defined as high titer of properly folded protein

# Products in Development and Pipeline Products (includes partnered products)

## Pipeline highlights



## Products in Development

### PF708: Therapeutic equivalent candidate to Forteo<sup>®</sup>, NDA accepted by FDA for review

- ▶ Forteo<sup>®</sup> (teriparatide) **rDNA peptide and pen combination product** for treatment of high fracture risk osteoporosis
- ▶ **Positive phase 3 study results** indicated no significant clinical or statistical differences between PF708 and Forteo
- ▶ **505(b)(2) NDA PDUFA goal date October 7, 2019**
- ▶ Potential **US commercial launch as early as Q4'19**, subject to FDA approval and other factors
- ▶ **Clear IP strategy** with no 30 month stay hurdle from Lilly on device patent
- ▶ **Global commercialization partnerships** established targeting \$1.7B anabolic osteoporosis market



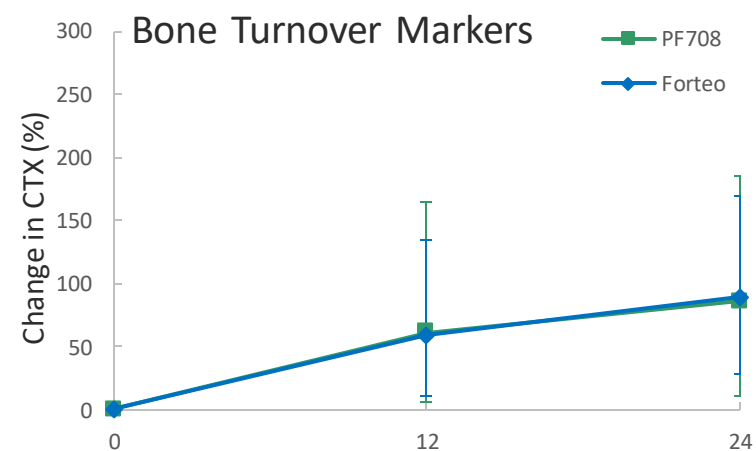
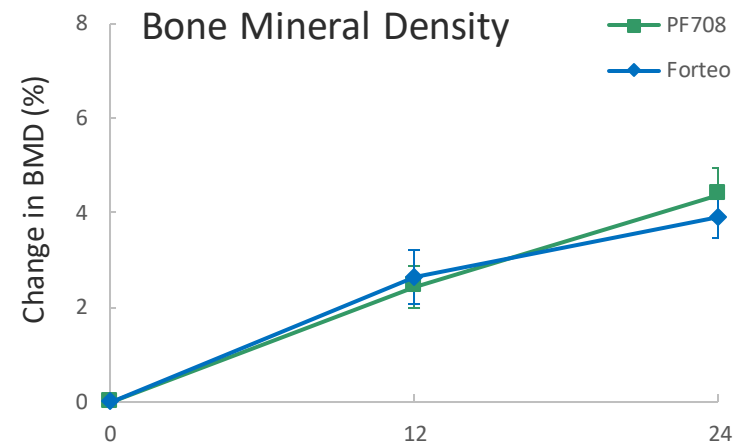
PF708 (teriparatide [rDNA] injection)



## PF708 Clinical

# Positive clinical results from clinical program

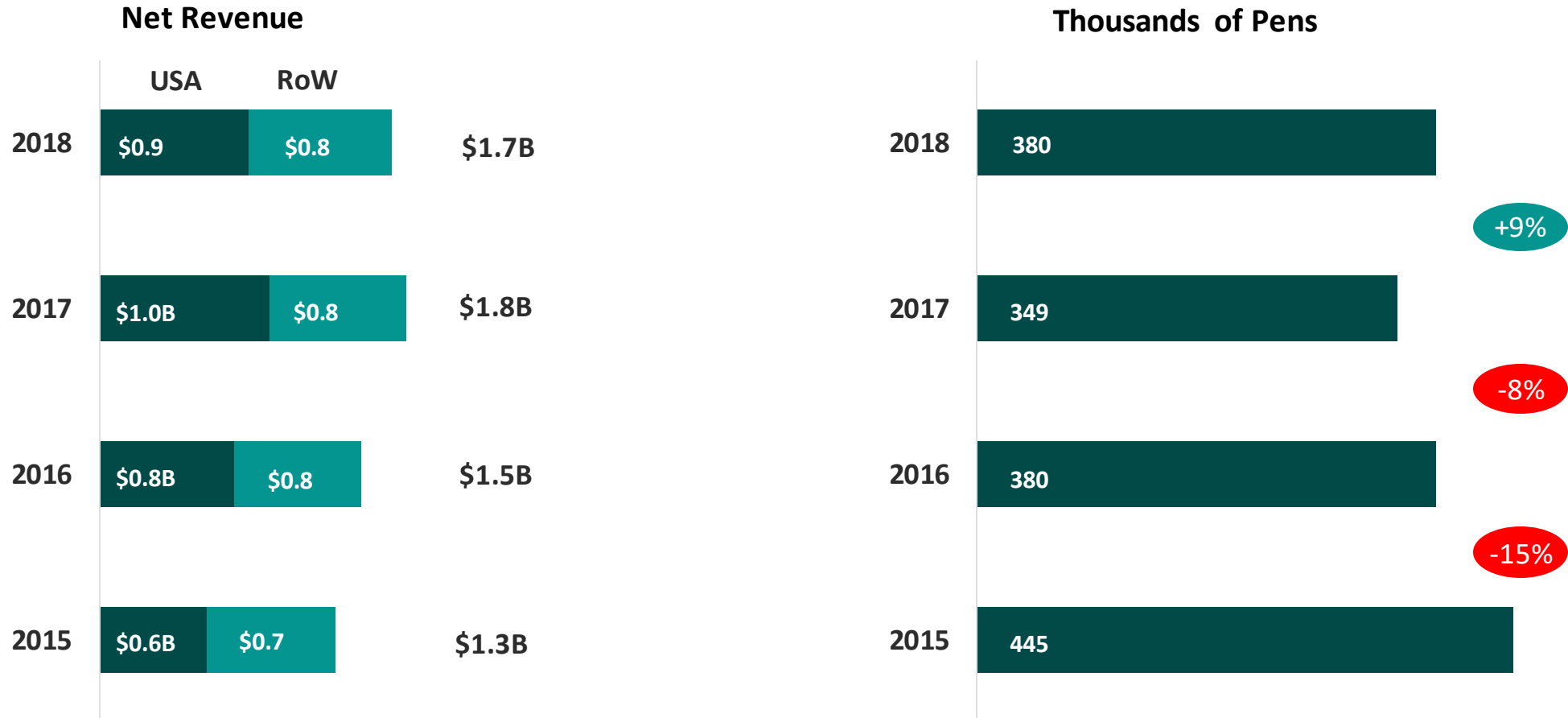
- ▶ Phase 3 (PF708-301) study comparing PF708 and Forteo in 181 osteoporosis patients demonstrated comparable overall profiles across multiple endpoints:
  - ▶ No imbalances in severity or incidence of adverse events
  - ▶ No clinically or statistically significant differences in immunogenicity, bone mineral density and bone turnover markers
- ▶ Phase 1 (PF708-101) study in healthy subjects demonstrated bioequivalence



# PF708 Commercial Outlook

## \$1.7B Opportunity - Resumption of volume growth driven by price elasticity

### Anabolic Osteoporosis Market\*



\* Source: Lilly and Radius filings for Forteo®, Forsteo and Tymlos®, IQVIA reports

# Potential US launch as early as Q4 2019, limited competition expected

## U.S. Patent landscape Forteo®

Expiry of 3  
Formulation  
Patents

Dec '18

Pfenex filed PIII  
against these

Expiry of 3  
Method of  
Treatment  
patents

Aug '19

45 day waiting  
period under  
Hatch-Waxman  
expired April 11,  
2019. No 30  
month stay from  
Lilly

Expiry of 1  
Device Patent

Mar '25

## Hurdles to entry

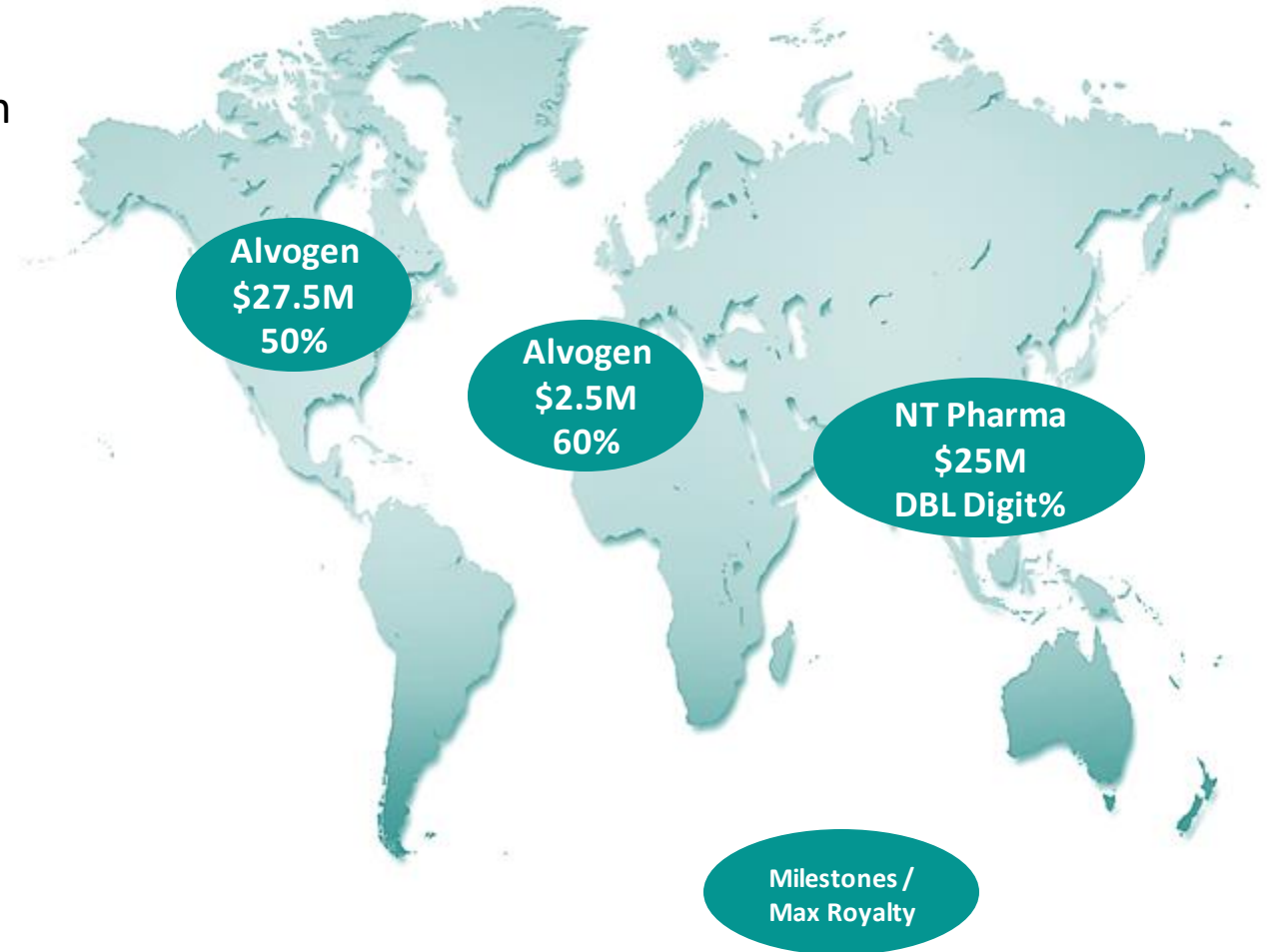
- Combination Product
- Recombinant v. Synthetic API
- Clinical Requirements
- Regulatory Path

**Very few potential competitors identified**

## PF708 Commercial Partnerships

### Commercial partnerships established in majority of global markets

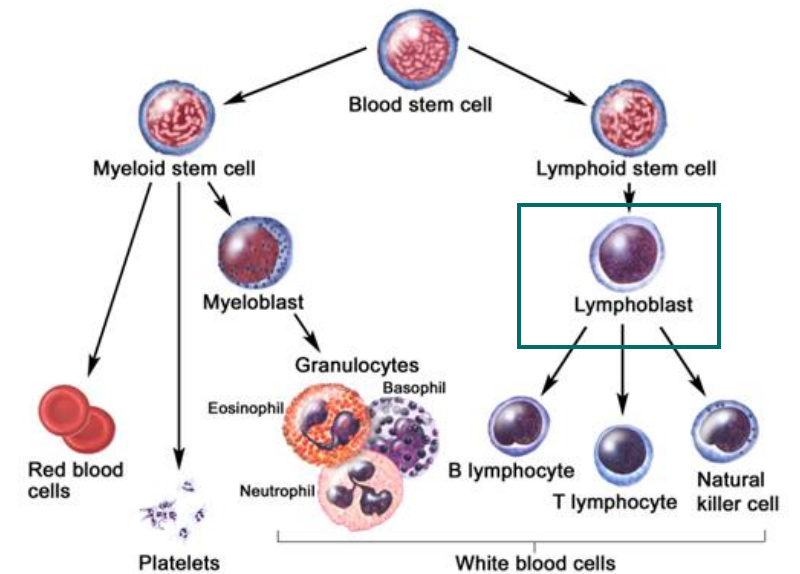
- ▶ **US – Alvogen** – 1.3B specialty Gx
  - Assumes responsibility and costs for commercial manufacturing and supply chain and commercialization
  - Proven successful in first to market litigation and launches
  - Robust manufacturing, supply chain and commercial organization
- ▶ **EU – Alvogen and Theramex** (ex-Teva woman's health)
- ▶ **MENA – Alvogen and SAJA** (current Forsteo distributor)
- ▶ **China + select Asia – NT Pharma**
- ▶ **ROW - Alvogen**



## Products in Development

# Jazz Pharmaceuticals/Pfenex partnership

- ▶ Applies Pfenex Expression Technology to the development of two therapeutic candidates for the treatment of Acute Lymphoblastic Leukemia (ALL)
- ▶ Pfenex granted Jazz worldwide rights to develop and commercialize multiple hematology/oncology products
  - ▶ PF743 recombinant crisantaspase
  - ▶ PF745 recombinant crisantaspase with half-life extension technology
- ▶ Key program highlights:
  - ▶ PF743 process development completed and PF745 process development on-going
  - ▶ Up to \$225M in upfront and potential milestone payments, plus tiered royalties on net sales
    - ▶ \$189M in milestones remains eligible to receive
    - ▶ Up to \$30M development, \$34M regulatory, \$125M sales

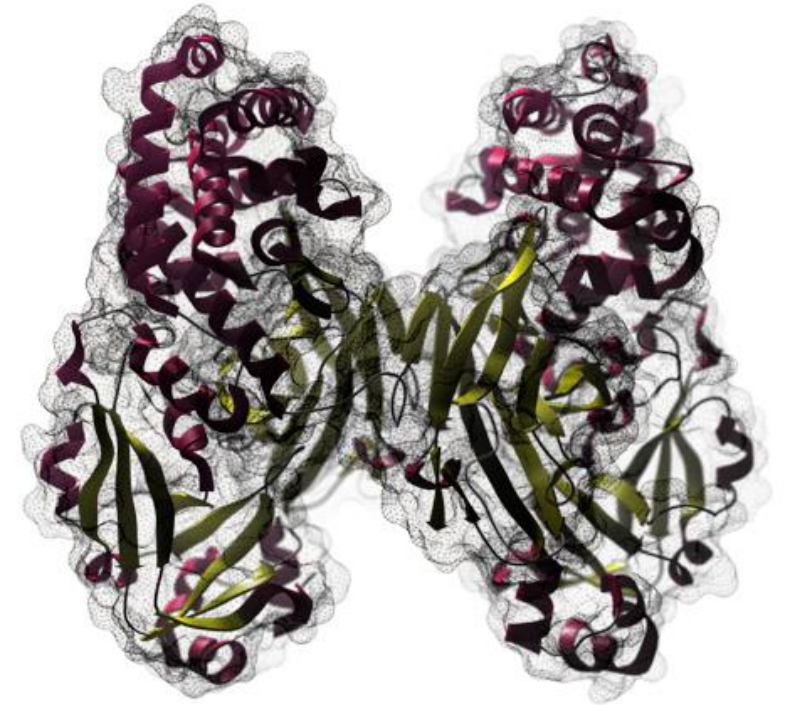


- Acute Lymphoblastic Leukemia (ALL) is a malignant cancer of the bone marrow and blood resulting in an abnormal number of immature white blood cells
- 6,000 patients diagnosed each year in the US, half of which are children under 14 years of age
- Most common form of pediatric cancer

## CRM197 Vaccine Business

### Pfenex CRM197 used in multiple late stage clinical vaccine product candidates

- ▶ CRM197 is a well characterized protein that functions as a carrier in vaccines to enhance immunogenicity
- ▶ Pfenex developed recombinant CRM197 produced by its platform
- ▶ Used by partners in multiple clinical stage product candidates for diseases including pneumococcal and meningitis bacterial infections
- ▶ Merck & Co. Inc. and Serum Institute of India (SII) are among companies with which Pfenex has royalty bearing development and commercial partnerships in place
- ▶ 2018 - Merck initiated multiple P3 studies of PCV-15 (V114), for the prevention of pneumococcal disease, granted Breakthrough Designation by FDA
- ▶ Q1'19 SII Pneumosil positive P3 data results and initiation of World Health Organization (WHO) prequalification process



## Pipeline Products

### PF582 and PF529 biosimilar candidates to Lucentis® and Neulasta®

- ▶ PF582 is our biosimilar candidate to Lucentis® (ranibizumab)
- ▶ Global Lucentis® market was approximately \$3.7 billion in 2018<sup>3</sup>
- ▶ Phase 1/2 first-in-human study completed:
  - ▶ Met primary objective of demonstrating similar safety and tolerability between PF582 and Lucentis®
  - ▶ Demonstrated consistent pharmacological activity between PF582 and Lucentis®
- ▶ PF529 is our biosimilar candidate to Neulasta® (pegfilgrastim)
- ▶ Neulasta® global sales in 2018 of \$4.5 billion<sup>4</sup>
- ▶ Production process developed and extensive analytical comparability to reference product completed
- ▶ US FDA feedback for PF529 supports the feasibility of development under the 351(k) biosimilar pathway

**Programs paused in Q4'17. Considering strategic partnership opportunities to maximize value**

# Experienced Team

## Established leadership aligned to drive growth

Senior Management



**Evert (Eef) Schimmelpennink**  
CEO, President, Director and Secretary



**Shawn A. Scranton**  
Chief Operating Officer



**Patrick K. Lucy**  
Chief Business Officer



**Susan A. Knudson**  
Chief Financial Officer



**Martin B. Brenner**  
Chief Scientific Officer



Board of Directors

**Jason Grenfell-Gardner**



**Robin Campbell**



**Philip Schneider**



**Sigurdur Olafsson**



**John Taylor**



**Magda Marquet**





# Investment Highlights



## Advanced Pipeline

Potential to commercialize PF708 as early as Q4'19, subject to FDA approval and other factors



## Distinct value drivers

- Up to \$280M+ in upfronts and potential milestones
- Sales and royalties as early as Q4'19, subject to FDA approvals and other factors

### PF708 Teriparatide

- Gx to Forteo®, \$1.7B anabolic osteoporosis market
- PDUFA goal date of Oct 7, 2019, potential U.S. launch as early as Q4'19
- Partnered globally: \$30M milestones, 50% of gross profits in U.S. if AP rated, 60% ex-U.S. pending geography

### PF743/PF745 Jazz Partnership

- Up to \$225M upfront and potential milestones, \$189M remains eligible to receive + royalties on sales
- PF743 process development completed
- PF745 process development on-going

### CRM197 Carrier Protein

- Used by various pharmaceutical companies in vaccine development
- Merck PCV-15 (V114) P3 on-going, SII Pneumosil positive P3 results, WHO prequalification process initiated
- Royalties on net sales



## Cash

Cash position of \$45.8M and 31.5M shares outstanding (as of 3/31/2019)



NYSE American: PFINX

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# References

<sup>1</sup> Based on publicly available 2018 sales data for the third party branded pharmaceutical company

<sup>2</sup><https://www.sec.gov/Archives/edgar/data/1367644/000136764416000084/form8kearningsq3q2016.htm>

<sup>3</sup> Roche Finance Report 2018 and Novartis Annual Report 2018

<sup>4</sup> Amgen 2018 10K