Rare Disease
Our discussions during Pfizer’s Investor Day include forward-looking statements about our anticipated future operating and financial performance, business plans and prospects; expectations for our product pipeline, in-line products and product candidates, including anticipated regulatory submissions, data read-outs, study starts, approvals, revenue contribution, growth, performance, timing of exclusivity and potential benefits; manufacturing and product supply; our efforts to respond to COVID-19, including our investigational vaccine candidate against SARS-CoV-2 and our investigational protease inhibitor, and our expectations regarding the impact of COVID-19; our ability to successfully capitalize on growth opportunities and prospects; plans for and prospects of our acquisitions and other business development activities, including our proposed transaction with Mylan N.V. (Mylan) to combine Upjohn and Mylan to create a new global pharmaceutical company; plans relating to share repurchases and dividends; and other statements about our business, operations and financial results that are each subject to substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Among other things, statements regarding revenue and earnings per share growth; the development or commercial potential of our product pipeline, in-line products, product candidates and additional indications, including expected clinical trial protocols, the timing of the initiation and progress of clinical trials and data read-outs from trials; the timing for the submission of applications for and receipt of regulatory approvals; expected breakthrough, best or first-in-class status, blockbuster status of our medicines or vaccines; and the impact of anticipated improvements to our clinical operation performance are forward-looking and are estimates that are subject to change and clinical trial and regulatory success. These statements are subject to risks, uncertainties and other factors that may cause actual results to differ materially from past results, future plans and projected future results. Additional information regarding these and other factors can be found in Pfizer’s Annual Report on Form 10-K for the fiscal year ended December 31, 2019 and in our subsequent reports on Form 10-Q, including in the sections thereof captioned “Risk Factors” and “Forward-Looking Information and Factors That May Affect Future Results”, as well as in our subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com. Potential risks and uncertainties also include the impact of COVID-19 on our sales and operations, including impacts on employees, manufacturing, supply chain, marketing, research and development and clinical trials. The forward-looking statements in these presentations speak only as of the original date of the presentation and we undertake no obligation to update or revise any of these statements. Today’s discussions and presentations are intended for the investor community only; they are not intended to promote the products referenced herein or otherwise influence healthcare prescribing decisions. All trademarks in today’s presentations are the property of their respective owners.
Rare Disease Leadership Team

Suneet Varma
Global President,
Rare Disease

Brenda Cooperstone, MD
Chief Development Officer,
Rare Disease

Seng Cheng, PhD
Chief Scientific Officer,
Rare Disease
Leading Innovation in Rare Disease Through Focused Strategy

A Leading In-Line Portfolio

- Rare Hematology
- Rare Endocrine/Metabolic
  - 7 Products in Clinical Development

Expand through Launch

- Rare Cardiology
- Rare Neurology
  - 3 Products in Clinical Development

Scale to Innovate & Accelerate

- End-to-End Capabilities in Gene Therapy
  - 3 Gene Therapy Manufacturing Sites
  - $800M Investment

>1,000 Colleagues Across ~100 Countries
Robust Rare Disease Pipeline With 6 New Molecular Entities in Phase 3 by Year End 2020

### In line
- **Rare Hematology**
  - Hemophilia B
  - Hemophilia A
  - Pan Hemophilia (Marstacimab)

- **Rare Endocrine/Metabolic**
  - Growth Hormone Deficiency (Somatrogon)

- **Rare Neurology**
  - Duchenne Muscular Dystrophy

- **Rare Cardiology**
  - LMNA-related Dilated Cardiomyopathy (p38 MAPK Inhibitor)

### Phase 3*
- **Rare Hematology**
  - Hemophilia A

### Phase 1 & 2
- **Rare Hematology**
  - Sickle Cell Disease (E-Selectin antibody)
  - Sickle Cell Disease (HbS Modulator)

- **Rare Endocrine/Metabolic**
  - Achondroplasia (Recifercept)

- **Rare Neurology**
  - ITP/CIDP** (IVIG Mimetic)

- **Rare Cardiology**
  - Wilson Disease

### Selected Pre-Clinical
- **Rare Hematology**
  - Hemophilia B
  - Hemophilia A
  - Pan Hemophilia (Marstacimab)

- **Rare Endocrine/Metabolic**
  - Growth Hormone Deficiency (Somatrogon)

- **Rare Neurology**
  - Duchenne Muscular Dystrophy

- **Rare Cardiology**
  - LMNA-related Dilated Cardiomyopathy (p38 MAPK Inhibitor)

**Notes:**
- *Phase 3 pending initiation for Hemophilia A and Duchenne Muscular Dystrophy*
- **Idiopathic thrombocytopenic purpura/chronic inflammatory demyelinating polyneuropathy**

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**Gene Therapy**

**Breakthroughs that change patients' lives**
Robust Rare Disease Pipeline With 6 New Molecular Entities in Phase 3 by Year End 2020

<table>
<thead>
<tr>
<th>In line</th>
<th>Rare Hematology</th>
<th>Rare Endocrine/Metabolic</th>
<th>Rare Neurology</th>
<th>Rare Cardiology</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
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<tr>
<td>Phase 3*</td>
<td>Hemophilia B</td>
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<td>Duchenne Muscular Dystrophy</td>
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<tr>
<th>Phase 1 &amp; 2</th>
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<th>ITP/CIDP** (IVIG Mimetic)</th>
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<td>Selected Pre-Clinical</td>
<td>Sickle Cell Disease (HbS Modulator)</td>
<td>Wilson Disease</td>
<td>Amyotrophic Lateral Sclerosis</td>
<td>Rare Cardiac Disorders</td>
</tr>
</tbody>
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* Phase 3 pending initiation for Hemophilia A and Duchenne Muscular Dystrophy
** Idiopathic thrombocytopenic purpura/chronic inflammatory demyelinating polyneuropathy
Growing Leadership in Rare Cardiology
Growing Leadership in Rare Cardiology

- Vyndaqel / Vyndamax for ATTR-Cardiomyopathy
- Approved US Q2 2019 / EU Q1 2020
- 25 global submissions completed; 9 more planned through 2021

- PF-07265803 for Dilated Cardiomyopathy with lamin A/C gene mutation
- Phase III study enrolling

- Gene therapy for rare cardiac disorders

_pF_
Driving Treatment and Disease Understanding, Early Diagnosis to Solidify Leadership in Transthyretin Amyloid Cardiomyopathy (ATTR-CM)

Tafamidis 80 mg Demonstrated 30% Greater Survival Benefit Compared with 20 mg

<table>
<thead>
<tr>
<th>Phase 3 ATTR-ACT Combined with Long-term Extension Study and 61mg</th>
<th>Median follow-up 51 months</th>
<th>Hazard ratio (95% CI)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Unadjusted</td>
<td></td>
<td>0.700 (0.501, 0.979)</td>
</tr>
<tr>
<td>Age adjusted</td>
<td></td>
<td>0.612 (0.433, 0.865)</td>
</tr>
<tr>
<td>NT-proBNP adjusted</td>
<td></td>
<td>0.639 (0.455, 0.898)</td>
</tr>
<tr>
<td>6MWT adjusted</td>
<td></td>
<td>0.701 (0.496, 0.991)</td>
</tr>
<tr>
<td>Age/NT-proBNP/6MWT adjusted</td>
<td></td>
<td>0.571 (0.395, 0.827)</td>
</tr>
</tbody>
</table>

Favors 80 mg Favors 20 mg

Commitment to Understanding Prevalence and Increasing Early Diagnosis

- Participants with Left Ventricular Hypertrophy of Unknown Etiology (N=1500)
  Study Initiated Q3 2018 in EU
- Participants with Heart Failure with Preserved Ejection Fraction (N=2000)
  Study Initiation Q4 2020
- ATTR-CM Suspect & Detect
- Artificial Intelligence (AI) Machine Learning Model

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PF-07265803\(^1\) – p38 MAPK Inhibitor in Development for LMNA-related Dilated Cardiomyopathy (DCM)

Disease Overview

100+ known **mutations in lamin A/C gene**

~70% have **cardiac death, heart transplant or major cardiac event** by age 45\(^2\)

No disease specific treatment options

US prevalence estimate **50K**\(^3\) (6% of I-DCM); ~10% diagnosed

- Loss of functional lamin proteins in the setting of LMNA DCM associated with p38 MAPK activation\(^4\)
- A potent and selective, oral small molecule inhibitor of the p38 MAPK pathway
- Normalizes left ventricular morphology and improves function in the LMNA\(^{N195K}\) mouse model\(^5\)

\(^1\) Previously ARRY-371797
\(^3\) Hershberger RE, et al. Nat Rev Cardiol. 2013 Sep;10(9).
Phase 3 Study Initiated in Patients with LMNA-related DCM Based on Encouraging Phase 2 Results

Phase 2\(^1\) Demonstrated Sustained Improvement on 6-Minute Walk Test Distance (6MWD)

Primary Endpoint: 69 meters mean absolute change from baseline at 12 weeks

Mean 6MWD at Baseline = 321 m

Ongoing Phase 3 Study

- Randomized, placebo-controlled 24-week study in patients with LMNA-related DCM (N=160)
- Primary endpoint: change from baseline in 6MWD at 12 weeks
- Currently 30 patients enrolled
- Top line results anticipated in 2023

\(^1\) C. MacRae, M.R.G. et al. European Heart Journal, Volume 37, Issue suppl_1, 1 August 2016.

Note: 12 patients enrolled. 8 patients that completed 48 weeks of treatment continued to receive study drug under a continuing treatment protocol. There were no deaths during the study.
An Industry Leading End-to-End Gene Therapy Platform

Brendan & Colleen
An Industry Leading End-to-End Gene Therapy Platform

Positioned to Launch Three Transformational Gene Therapies by 2023

Discover
- 10 preclinical rAAV programs in rare cardiology, hematology, neurology & metabolic diseases
- Partnerships & acquisitions to complement internal pipeline
- First to manufacture rAAV at 2,000L bioreactor scale using HEK293 cells

Develop
- Three Phase 3 programs expected in 2020
- Extensive experience enabling acceleration
- Clinical trial site footprint supports global gene therapy trials
- Early and active engagement of patients and regulatory authorities

Deliver
- $800M investment in in-house manufacturing which aims to fully supply global demand
- Leader in shaping global policy
- Innovative solutions to address access and affordability challenges
Sustained expression of mean steady-state FIX activity of ~20% into year 4 with 5E11 vg/kg dose

Phase 1/2a and Long-Term Follow-Up (LTFU)
- 15 patients with follow-up of up to 4.5 years
- Mean ABR^3 of <1 & AIR^4 of ~1
- No treatment related SAEs

Phase 3
- Prospective lead-in study fully enrolled
- Pivotal first subject dosed in 2019
- Pivotal trial readout planned for 2021

WW prevalence ~88K; US/JP/EU ~17K^1
Projected 2022 Global Market Size ~$2B^2
FDA Breakthrough, RMAT & EMA Prime designations

Data from Phase 1/2a and LTFU studies, data cut July 2020, ABR data from March 2020 but available data suggests insignificant change
Giroctocogene fitelparvovec: Potential Best-in-Class Gene Therapy for Hemophilia A Patients

**Phase 1/2**
- 11 patients across 4 doses with up to 85 weeks of follow-up at Phase 3 dose
- Mean ABR$^3$ of 0 & AIR$^4$ of 0
- 1 SAE that resolved within 24 h; ALT elevations managed with steroids

**Phase 3**
- Prospective lead in trial started 2019
- Planning to dose first subject in 2020
- Pivotal trial readout planned for 2022

**Projected 2023 Global Market Size ~$11B**

**WW prevalence ~400K; US/JP/EU ~78K**

**Mean FVIII activity 71% (CA) from week 9-52 with 3E13 vg/kg dose**

Patients with data beyond 52 weeks show consistent FVIII levels

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3 ABR: annualized bleeding rate;
4 AIR: annualized infusion rate beyond protocol defined allowance of prophylaxis.
DMD Gene Therapy: No SAEs Observed in Additional 9 Boys Dosed

- Phase 1b
  - Dosed additional 9 boys at high dose (2E14 vg/kg) since ASGCT (18 total patients), 3 with drug from commercial manufacturing process
  - No SAEs observed with modified immunomodulatory and monitoring regimen

- Phase 3
  - Pivotal trial in ambulatory patients planned first subject dose in 2020
  - Interim analysis anticipated in 2022

Preliminary signs of efficacy include mini-dystrophin expression, reduced serum CK and fat fraction on MRI, and improved NSAA scores

**WW prevalence ~140K; US/EU ~30K**

Projected 2023 Global Market Size ~$4B

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1 Crisafiulli et al. Orphanet Journal of Rare Diseases, 2020, 15:141.
3 2E14 vg/kg by the transgene method is approximately equivalent to 3E14 vg/kg by the ITR method, used in the initial part of the study.
Gene Therapy for Wilson Disease IND Planned in 2020

Widespread Decrease in Copper in VTX-801-treated Liver

Dose-dependent Restoration of Normal Biliary/Fecal Copper Excretion

Diagnosed prevalence ~21K US/EU
Projected 2026 Global Market Size ~$500M

- Wilson disease is a chronic, life-threatening disease caused by aberrant accumulation of copper in liver, brain and other vital organs
- VTX-801 (Vivet Therapeutics), an AAV vector encoding ATP7B, restores copper homeostasis and reverses liver pathology in mouse model of Wilson disease
- IND submission anticipated in 2020
- BLA submission planned for 2025

2 EvaluatePharma Aug 2020
Leader in Gene Therapy Manufacturing – Building for Growth

<table>
<thead>
<tr>
<th>Grade and Scale details</th>
<th>Grade</th>
<th>Scale</th>
</tr>
</thead>
<tbody>
<tr>
<td>~300,000 sq ft with Capacity to Fully Supply All Gene Therapy Programs in Pipeline, including DMD at Launch</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research &amp; Development</td>
<td>GLP</td>
<td>10-250L</td>
</tr>
<tr>
<td>Early Clinical ~80,000 sq ft</td>
<td>GMP</td>
<td>2 x 500L</td>
</tr>
<tr>
<td>Pivotal Clinical &amp; Commercial ~40,000 sq ft</td>
<td>GMP</td>
<td>3 x 2,000L</td>
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<tr>
<td>Commercial &gt;170,000 sq ft</td>
<td>GMP</td>
<td>8 x 2,000L</td>
</tr>
</tbody>
</table>

Kit Creek, NC
Durham, NC
Sanford, NC

Kit Creek, NC
Durham, NC
Sanford, NC
Framing the Future Opportunity

Michael
# Significant Clinical and Commercial Opportunities: Rare Cardiology

## LMNA-related Dilated Cardiomyopathy (p38 MAPK Inhibitor)

<table>
<thead>
<tr>
<th>50K</th>
<th>US total prevalence&lt;sup&gt;1&lt;/sup&gt;</th>
</tr>
</thead>
<tbody>
<tr>
<td>~35%</td>
<td>Peak Diagnosis Rate (7-10% at launch)</td>
</tr>
<tr>
<td>40-45%</td>
<td>Treatment Eligible&lt;sup&gt;2&lt;/sup&gt;</td>
</tr>
<tr>
<td>50-70%</td>
<td>Access (peak)</td>
</tr>
<tr>
<td>1</td>
<td>Near Term Competitor (at launch)</td>
</tr>
<tr>
<td>$0.5-1B</td>
<td>Potential global annual peak revenues</td>
</tr>
</tbody>
</table>

## Vyndaqel/Vyndamax

<table>
<thead>
<tr>
<th>100K</th>
<th>US total prevalence&lt;sup&gt;3&lt;/sup&gt;</th>
</tr>
</thead>
<tbody>
<tr>
<td>15%</td>
<td>Current Diagnosis Rate (1% at launch, &gt;35% peak)</td>
</tr>
<tr>
<td>~85%</td>
<td>Seek Treatment</td>
</tr>
<tr>
<td>~60%</td>
<td>Access (current)</td>
</tr>
<tr>
<td>0</td>
<td>Near Term Competitor (when launched)</td>
</tr>
<tr>
<td>$&gt;1B</td>
<td>Potential 2020 Sales</td>
</tr>
</tbody>
</table>

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2. Includes NYHA Class II/III patients with an ICD or CRT implant.
3. PFE Internal Analysis.
# Significant Clinical and Commercial Opportunities: Gene Therapies

<table>
<thead>
<tr>
<th>Hemophilia B</th>
<th>Hemophilia A</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>~17K</strong> US / JP / EU total prevalence&lt;sup&gt;1&lt;/sup&gt;</td>
<td><strong>~78K</strong> US / JP / EU total prevalence&lt;sup&gt;1&lt;/sup&gt;</td>
</tr>
<tr>
<td><strong>60-65%</strong> Target Population (moderate &amp; severe)</td>
<td><strong>60-65%</strong> Target Population (moderate &amp; severe)</td>
</tr>
<tr>
<td><strong>~30%</strong> Eligibility&lt;sup&gt;2&lt;/sup&gt;</td>
<td><strong>~30%</strong> Eligibility&lt;sup&gt;2&lt;/sup&gt;</td>
</tr>
<tr>
<td><strong>&gt;80%</strong> Access (peak)</td>
<td><strong>&gt;80%</strong> Access (peak)</td>
</tr>
<tr>
<td><strong>1-2</strong> Near Term Competitors</td>
<td><strong>2</strong> Near Term Competitors</td>
</tr>
<tr>
<td><strong>$0.5-1B</strong> Potential global peak revenue</td>
<td><strong>&gt;$1B</strong> Potential global peak revenue</td>
</tr>
</tbody>
</table>

2. Gene therapy is expected to have clinical eligibility requirements such as a lack of neutralizing antibodies and liver disease. Hemophilia gene therapy is expected to require moderate or severe disease and an absence of known history of inhibitors. Hemophilia gene therapy is expected to be available for adults initially, and for a broad age range over time. Patient intent to seek treatment is included.
## Significant Clinical and Commercial Opportunities: Gene Therapies

<table>
<thead>
<tr>
<th>Duchenne Muscular Dystrophy</th>
<th>Wilson Disease</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>30K</strong> US / EU total prevalence&lt;sup&gt;1&lt;/sup&gt;</td>
<td><strong>21K</strong> US / EU total prevalence&lt;sup&gt;4&lt;/sup&gt;</td>
</tr>
<tr>
<td><strong>56%</strong> Target Population (boys ages 4-18)&lt;sup&gt;2&lt;/sup&gt;</td>
<td><strong>15-20%</strong> Target Population (failing therapy plus those transitioning from stable-to-fail)</td>
</tr>
<tr>
<td><strong>75-85%</strong> Eligibility&lt;sup&gt;3&lt;/sup&gt;</td>
<td><strong>65-70%</strong> Eligibility&lt;sup&gt;3&lt;/sup&gt;</td>
</tr>
<tr>
<td>&gt;75% Access (peak)</td>
<td>&gt;80% Access (peak)</td>
</tr>
<tr>
<td>1 Near Term Competitor</td>
<td>1 Near Term Competitor</td>
</tr>
<tr>
<td>&gt;$2B Potential global annual peak revenues</td>
<td>&lt;$0.5-1B Potential global annual peak revenues</td>
</tr>
</tbody>
</table>

2. Of the total prevalence, 31% are ambulatory boys aged 4-18 and 25% are non-ambulatory boys aged 4-18.
3. Gene therapy is expected to have clinical eligibility requirements such as absence of neutralizing antibodies.
Our Robust Pipeline Aims to Make a Profound Impact on Pfizer and the Lives of the Patients We Serve

2019/2020

Vyndaqel

Pfizer Rare Disease 1H 2020 Revenue of $1.3B, +36%G*

* Operational growth excluding FX
Our Robust Pipeline Aims to Make a Profound Impact on Pfizer and the Lives of the Patients We Serve

<table>
<thead>
<tr>
<th>2019/2020</th>
<th>2021</th>
<th>2022</th>
<th>2023</th>
<th>2024</th>
<th>2025</th>
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<td></td>
<td>Wilson Disease**</td>
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Poised for at Least 1 Launch per Year*

* On Average
** Expected BLA filing in 2025