



**Innovating to Accelerate the Delivery of Transformative Therapies to Patients**

**PQRI/FDA Conference on Advancing Product Quality April 9, 2019**  
**Stephanie Krogmeier**



YOUR  
COMFORT  
ZONE



WHERE THE  
MAGIC HAPPENS

[WWW.THEIRONYOU.COM](http://WWW.THEIRONYOU.COM)



# Three Essential Components of Innovation

- *Commit to a purpose*

- *Be brave*

- *Be different*



# Our Vision

Vertex is a global biotechnology company that invests in **scientific innovation** to create **transformative medicines** for people with **serious diseases** with a focus on **specialty markets**



# Our Mission and Values

*Innovate to Redefine Health and Transform  
People's Lives with New Medicines*

*Uncompromising  
**Commitment**  
to Patients*

***Innovation**  
Is Our Lifeblood*

***Fearless**  
Pursuit of Excellence*

***“We”**  
Wins*



# Innovation is our Lifeblood

## VERTEX'S COMMITMENT TO RESEARCH & DEVELOPMENT

We believe scientific innovation is the key to creating value. Bringing together the brightest minds, investing in science and taking smart risks, we strike at the core of serious diseases to transform the lives of people around the world.

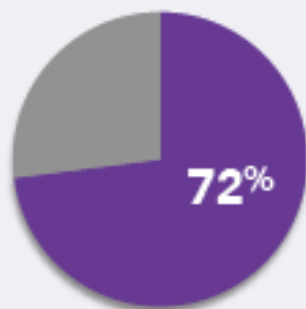


### OUR FOCUS

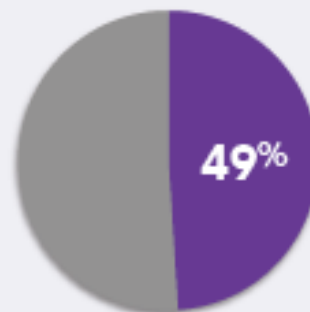
*We invest the vast majority of our resources in our innovation engine.*

### BUSINESS OPERATING COSTS

(2013 - 2017)



VERTEX



PEER COMPANY AVG<sup>1</sup>



### OUR PEOPLE



**3 OUT OF 5**  
VERTEX EMPLOYEES  
ARE DEDICATED TO  
**RESEARCH &  
DEVELOPMENT**



# Vertex's Noteworthy Industry Firsts

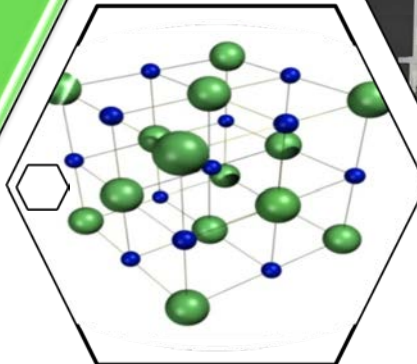


First two  
Breakthrough  
Therapy  
Designations  
from U.S.  
FDA

1<sup>st</sup> Approved  
CM Process  
with RTRT

1<sup>st</sup> Full  
Quality by  
Design  
Submission  
(Incivek)

1<sup>ST</sup> Approved  
SDD Product  
(Incivek)





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“Traditional manufacturing...could delay acceleration of development under the...”

“*Fixing  
U.S.  
com  
trea*

“The innovator advantage is not just drug discovery, it is also with manufacturing. The pharmaceutical industry (unlike many other industries) does not often see innovation in manufacturing as a key strategic advantage. The few firms who do, will see immense value in prioritizing this.” – Dr. Janet Woodcock

“No matter how good your science is, and how important the unmet need, approval also hinges on whether you have a manufacturing facility ready to go and a plan in place for scaling up production so you can manufacture your new drug...”



# CMC Challenges for Expedited Development and Review

Commercial manufacturing process and formulation need to be developed early to align with the accelerated clinical development program

Scale up issues need to be addressed to ensure availability of quality product at the time of approval

A plan for validation and stability studies needs to be developed to keep these activities off critical path to approval

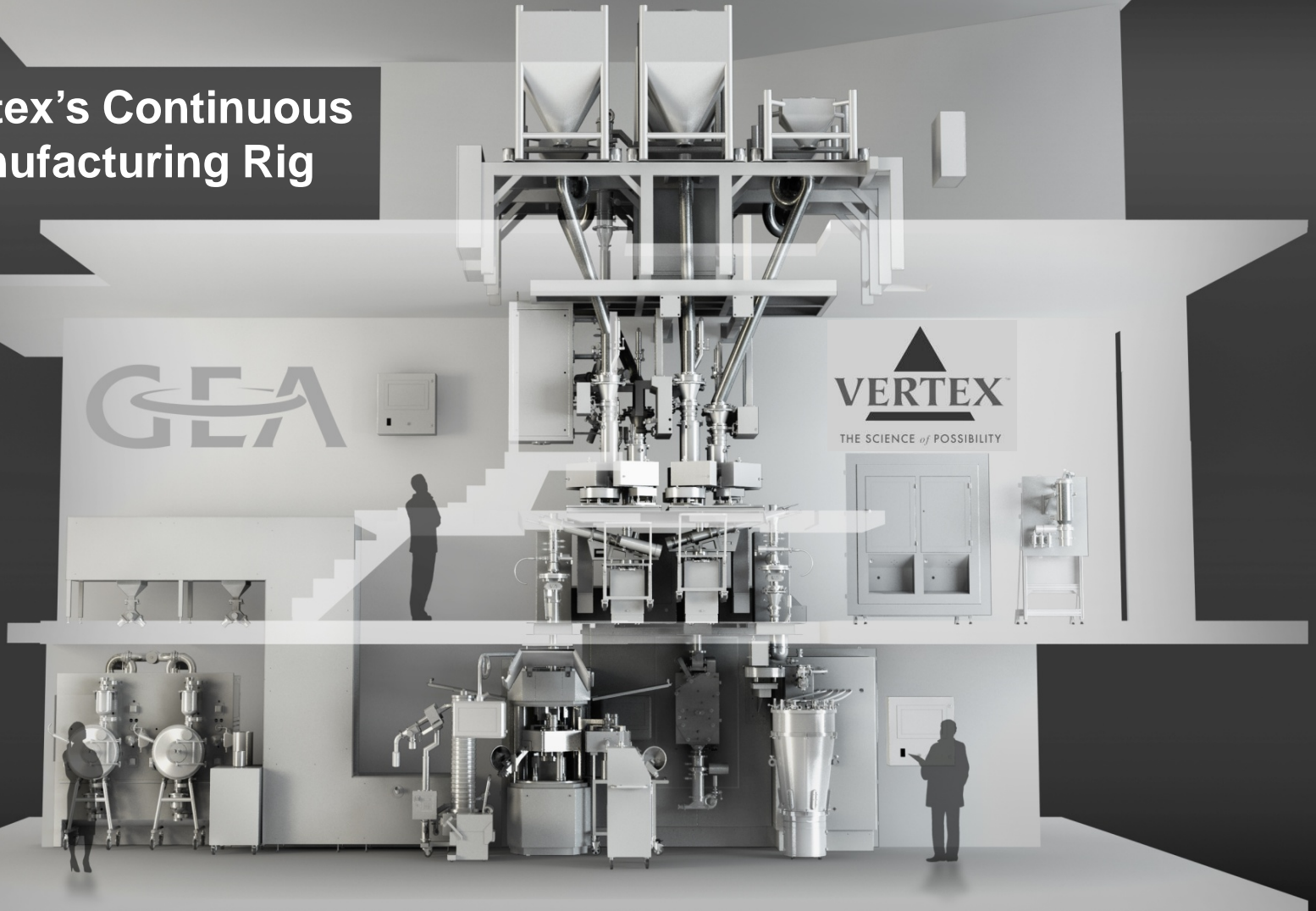
**CMC Challenges for Expedited Development are Valid**



# Vertex's Continuous Manufacturing Rig

GEA

**VERTEX**  
THE SCIENCE OF POSSIBILITY



# Vertex's Continuous Manufacturing Rig

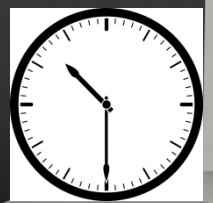
Powder In



Day 1 AM

Much Smaller Footprint

- Smaller scale equipment
- All unit ops in one facility



Day 1 AM



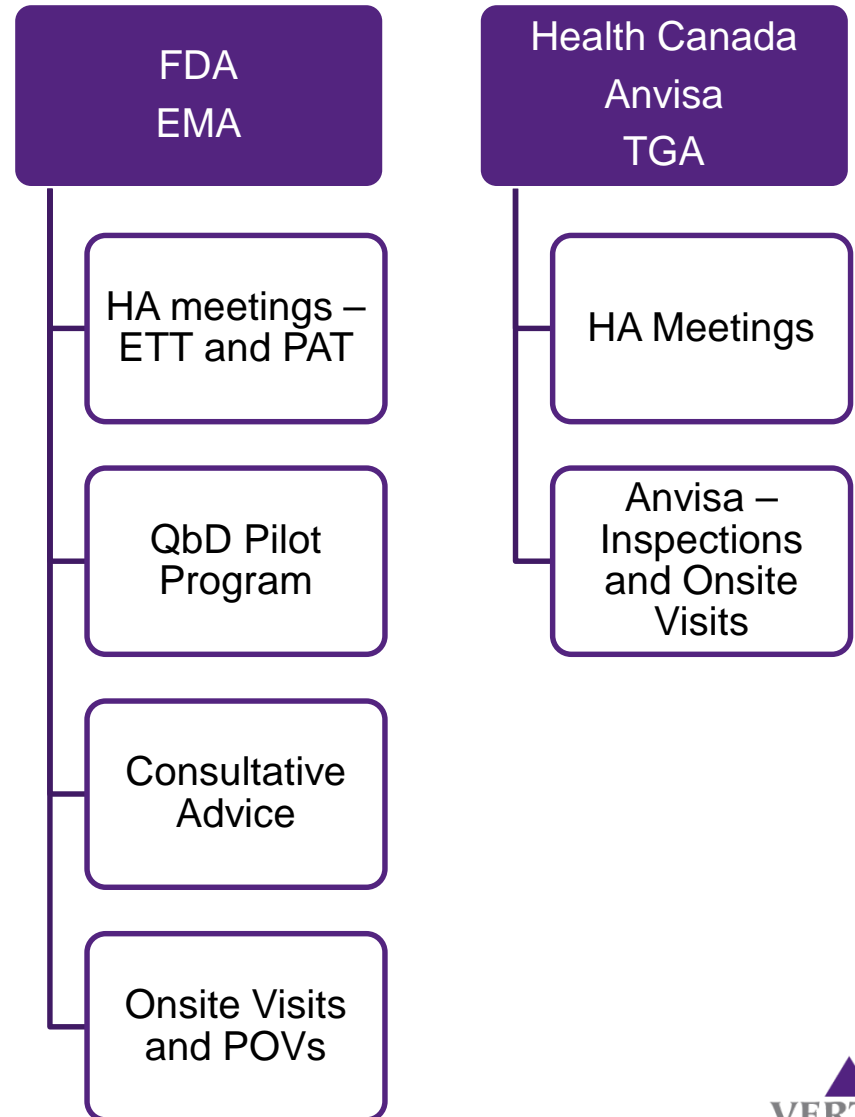
Coated Tablets Out

PAT Based Control Strategy => IPCs + RTRT



# Health Authority Interactions Opportunities

- Innovation in a regulated environment requires global health authority alignment
- Communication and transparency are critical for building relationships and establishing partnerships with Health Authorities
- Use all available options and avenues to interact with Health Authorities early and often



# From Business Drivers to CM Realization



Full support  
from the  
highest levels  
of  
management

CM central to  
development  
strategy to get  
breakthrough  
therapies to  
patients

Highly  
motivated and  
dedicated  
team

# Three Essential Components of Innovation

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**In order to be  
irreplaceable one  
must always  
be different.**

*Coco Chanel*

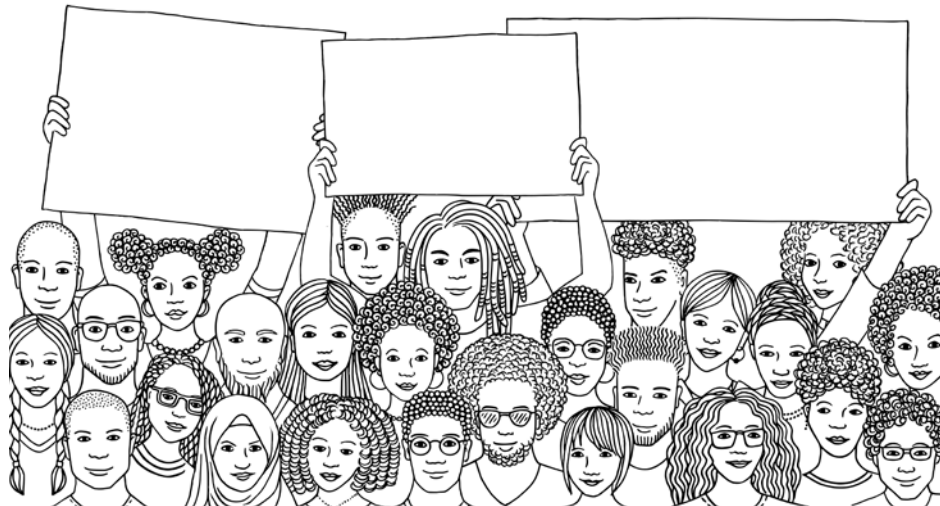


 **BrainyQuote**<sup>®</sup>

  
**VERTEX**

# Turns out.....

- In order to be successful one must always be *different*
- McKinsey found that companies with more diverse workforces perform better financially



# Diversity is Different

## Inherent

- Gender
- Race
- Age
- Religious background...

## Acquired

- Cultural fluency
- Global mindset
- Language skills
- Broad life experiences ...



Biotech  
Companies  
Founded

1000's

# Of all biotech companies, how many have internally discovered & developed four or more approved medicines (NMEs)?

IPOs  
790

≥1 approved  
medicine

59



≥2 approved  
medicines

18



≥4 approved  
medicines

5



**Genentech**

Est. 1976  
15,000 FTE



**AMGEN**

Est. 1980  
19,000 FTE



**GILEAD**

Est. 1987  
8,000 FTE



**REGENERON**

Est. 1988  
5,000 FTE



**VERTEX**

Est. 1989  
2,000 FTE



Sources: Nasdaq, MorganStanley,  
Pharmaprojects, EvaluatePharma, GlobalData



# Acknowledgements

- Vertex Team
  - Pharmaceutical Sciences
  - Technical Operations
  - Supply Chain Management
  - Quality
  - CMC Regulatory
- Our CMOs, Suppliers, and Research Collaborators



# Questions

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***Innovate to Redefine Health and Transform People's Lives with New Medicines***

