Huntington's Disease and Vertex Pharmaceuticals

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2010 Huntington's Disease Society of America

Potential new therapies for HD session

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Overview

- Background on Vertex Pharmaceuticals
- Why is Vertex interested in Huntington's Disease?
- The Vertex approach for HD

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Vertex Pharmaceuticals Incorporated

Small molecule drugs for serious diseases

- Founded in 1989
- 1300+ employees
- 5 R&D sites with stand-alone drug discovery capabilities

San Diego, CA
Pain, Cystic Fibrosis & Huntington's disease
HTS Screening and Automation

Cambridge, MA
Immune Mediated Inflammatory Diseases
Anti-infectives
Cancer
Biochemistry, Biomarkers
Clinical Development
Pharmaceutical Development & Operations

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ViroChem Pharma

Laval, Canada
Anti-infectives

Milton Park, UK
X-ray Crystallography
Immune Mediated Inflammatory Diseases
Cancer

Coralville, IA
Tuberculosis
Anti-microbials
Clinical Virology

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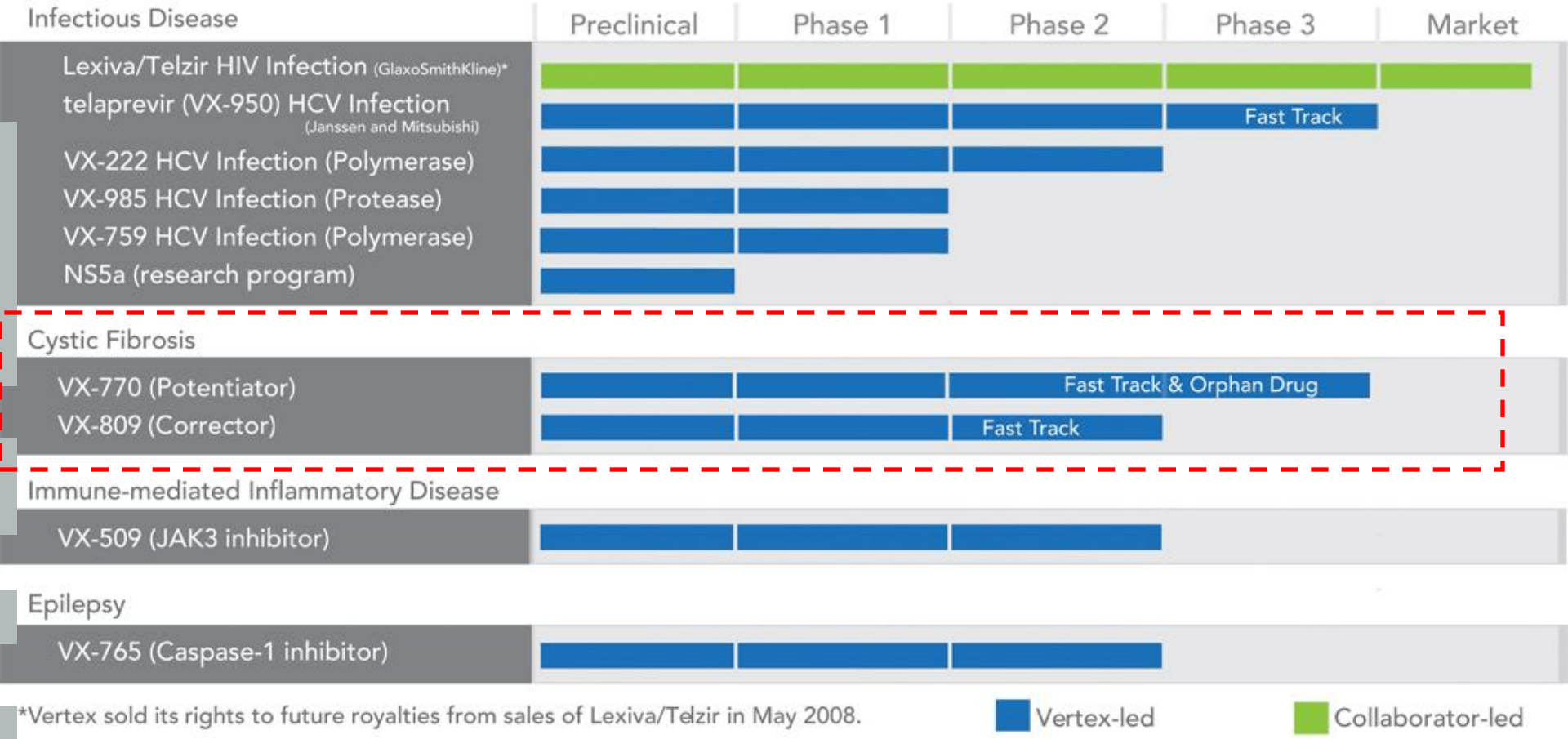
Vertex Development Pipeline



Vertex may be “Goldilocks” partner in Huntington’s disease

- Not too big to ignore HD due to market size
- Not too small to fully develop drugs on our own

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Successful collaboration with CFF to develop therapeutics for cystic fibrosis

- Collaboration between Vertex and the Cystic Fibrosis Foundation (1998)
  - Building high-throughput screens
- By 2007, two Vertex compounds in clinical trials
  - May 2008: VX-770 showed clinical efficacy in CF patients
  - Feb 2010: VX-809 showed effect on clinical biomarker in CF patients
  - 2 compounds demonstrated proof-of-concept for correcting molecular defect
- First-in-class therapeutics with potential to modify the disease course and not just treat symptoms

Cystic Fibrosis Foundation

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Similarities between CF and HD

	Cystic fibrosis	Huntington's disease
Inherited disease	Yes (single gene)	Yes (single gene)
Unmet medical need	High	High
Disease modifying therapies possible	Yes	Yes
Patient size	30,000 in US	30,000 in US
Volunteer health org and advocacy base	Strong	Strong

Goal: Utilize our experience in CF to serve as a blueprint for HD drug discovery efforts

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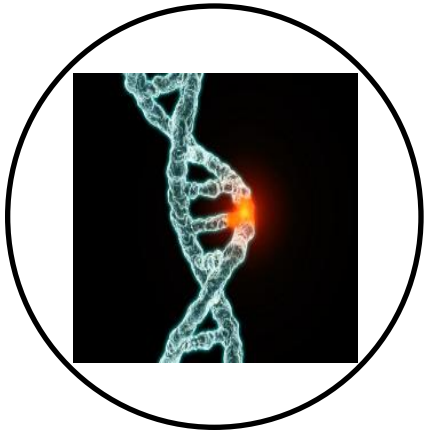
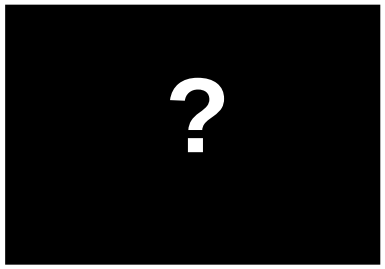
Overview of the Vertex HD Program

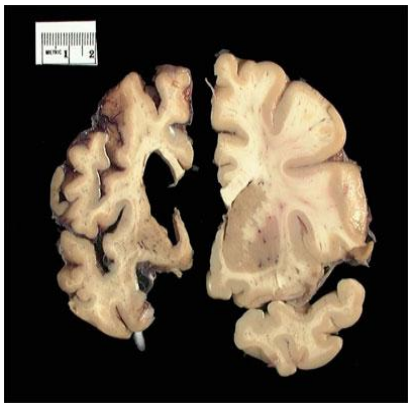
- Goal: Develop disease modifying therapies that delay the onset and progression of Huntington's Disease and improve the quality of patients' lives
- Focused on two scientific approaches to potential therapeutics for HD
  - directly targeting the core defect
  - Neuro-repair

Core Defect → **Molecular pathology** → **Degeneration**



Mutant htt





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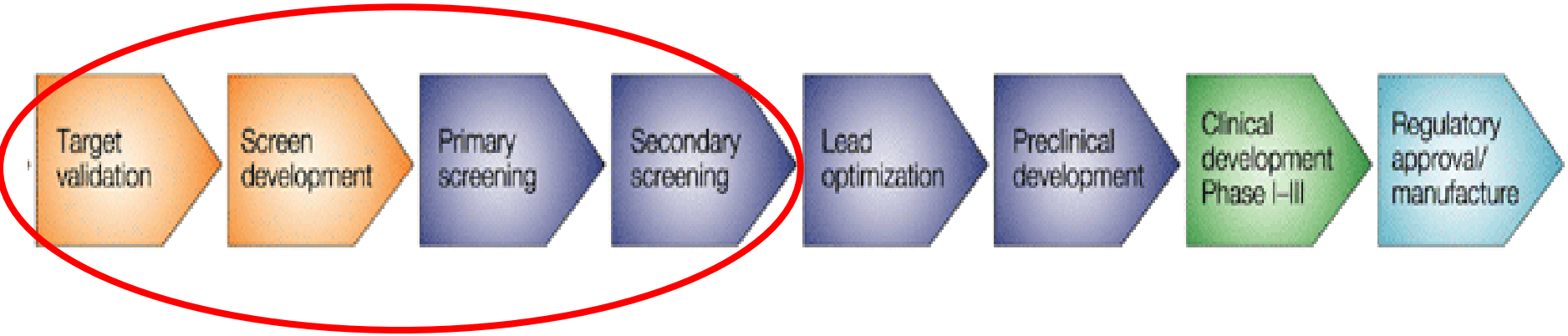
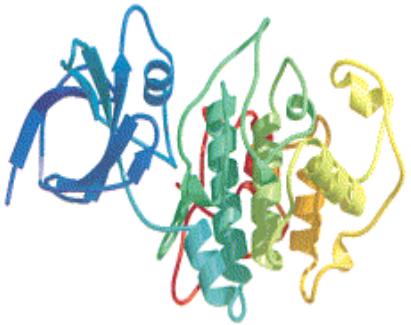
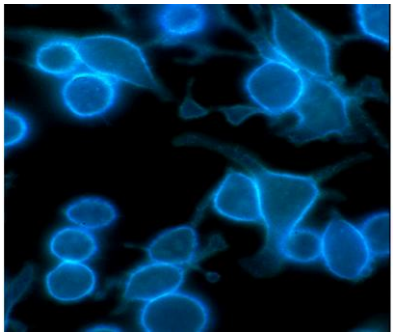
Vertex and HD: Moving forward



- Attacking HD by focusing on disease modifying therapies
- Building networks with scientific and clinical experts in the field of HD
- Commitment to translating scientific breakthroughs into improved patient care





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