

Huntington's Disease and Vertex Pharmaceuticals

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www.vrtx.com



Outline

- Background on Vertex Pharmaceuticals
- Vertex drug discovery collaboration with Cystic Fibrosis Foundation
- Why is Vertex interested in Huntington's disease?
- Vertex collaboration with CHDI on Huntington's disease

Vertex Pharmaceuticals Incorporated



Small molecule drugs for serious diseases

- Founded in 1989
- 1200+ employees
- 4 R&D sites with stand-alone drug discovery capabilities
 - Cambridge, MA, USA
 - San Diego, CA, USA
 - Milton Park, UK
 - Coralville, IA, USA



Cambridge, MA

Immune Mediated Inflammatory Diseases
Anti-infectives
Cancer
Biochemistry
Clinical Development
Pharmaceutical Development & Operations



San Diego, CA

Ion channels and Receptors
Cystic Fibrosis & Neurodegenerative Diseases
HTS Screening and Automation



Milton Park, UK

X-ray Crystallography
Immune Mediated Inflammatory Diseases
Cancer



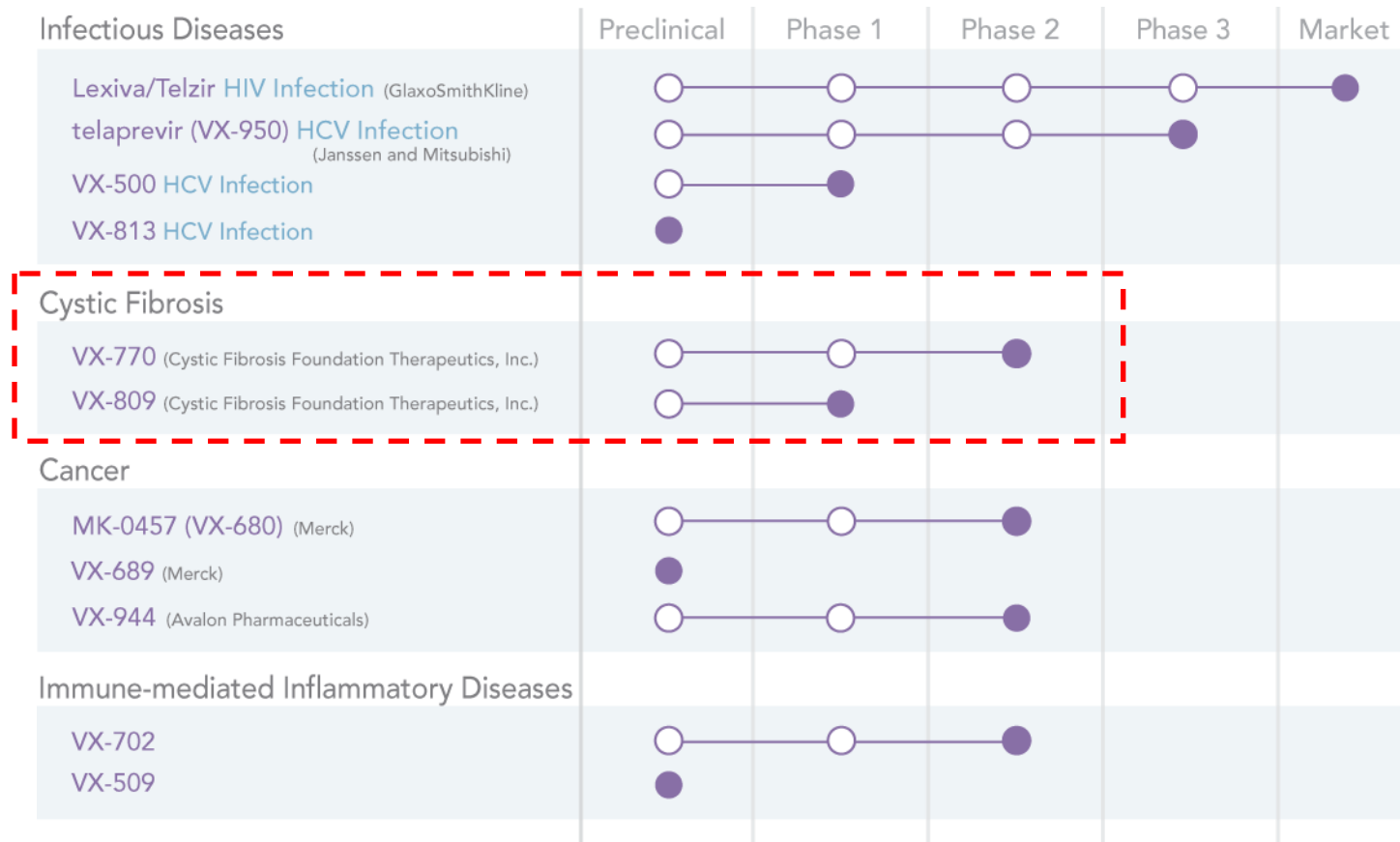
Coralville, IA

Tuberculosis
Anti-microbials
Clinical Virology

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Vertex Drug 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



Highly successful collaboration with Cystic Fibrosis Foundation



- Collaboration between Vertex and the Cystic Fibrosis Foundation to discover drugs targeting the molecular defect in CF
- Collaboration began in 1998 with high throughput screen; by 2007 Vertex had two compounds in clinical trials
 - May 2008: VX-770 showed clinical efficacy in CF patients
 - Demonstrated proof-of-concept for correcting the molecular defect in CF with a drug → showed that VX-770 is acting on the intended target and is providing a clinical benefit
- Establishing new paradigm for industry:non-profit organization collaboration in drug discovery

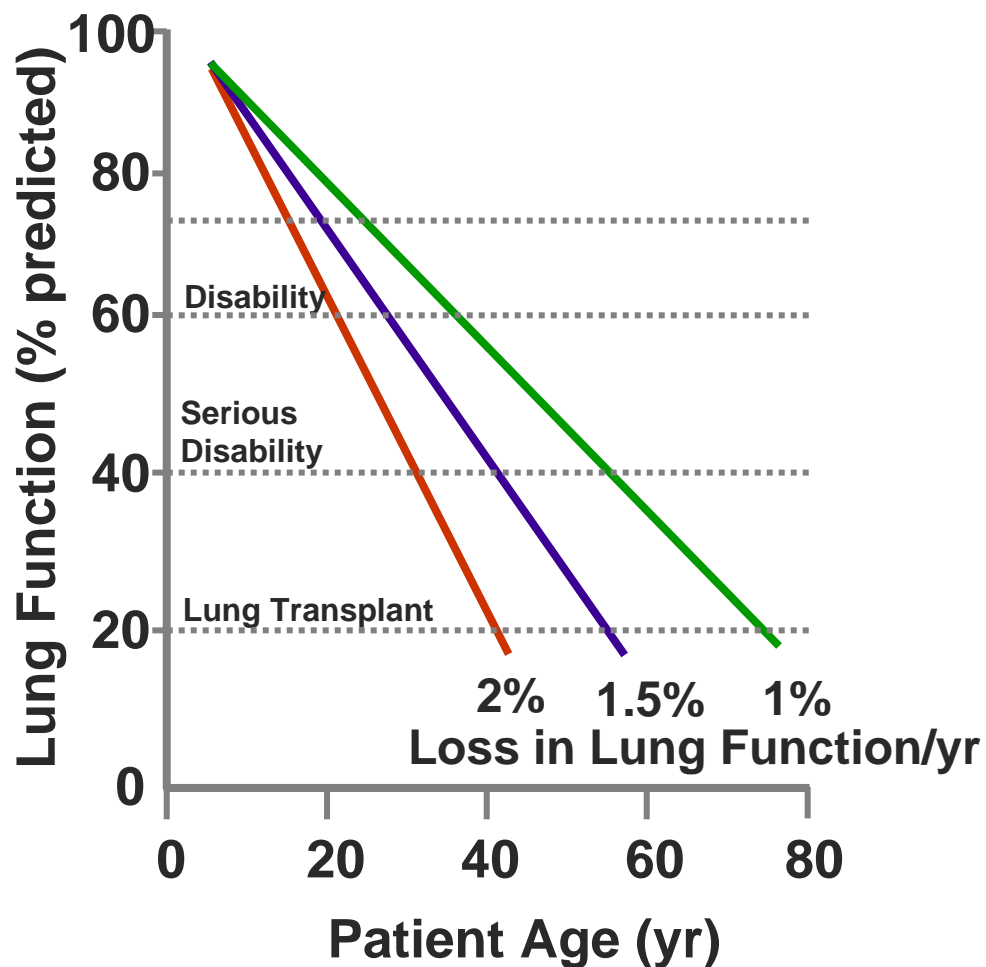
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Intervene early with disease-modifying approaches in CF

Reducing Loss of Lung Function by 1%/yr Could Double Life Span



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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
Vol health org and advocacy base	Strong	Strong

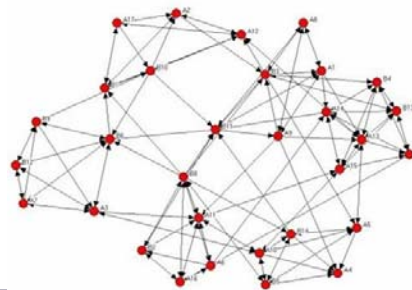
Why is Vertex interested in Huntington's disease?

- High unmet medical need
- Maturing scientific understanding of the disease
- Readily identified and motivated patient base
- Possibility for smart clinical trials
- Vertex has expertise in developing drugs to fix broken proteins (CF)
- Replicate successful CF collaborative network model in HD

Vertex-CHDI collaboration in HD



- Vertex and CHDI research collaboration initiated in April 2008
- Focused on two scientific approaches to potential therapeutics for HD
 - Protein folding: repairing mutant huntingtin
 - Stem cells: repairing nerve cell loss in HD
- Phased exploration makes sense for both CHDI and Vertex
 - Initially two years, could be extended upon positive results
- CHDI and other organizations bring a network of researchers, clinicians and patients



Vertex and HD

- Entry into neurodegenerative diseases via CHDI collaboration
- Vertex brings strong expertise in cellular assays and protein folding
- Commitment to translating scientific breakthroughs into improved patient care

