

#### **Overview of Medivation and Dimebon**

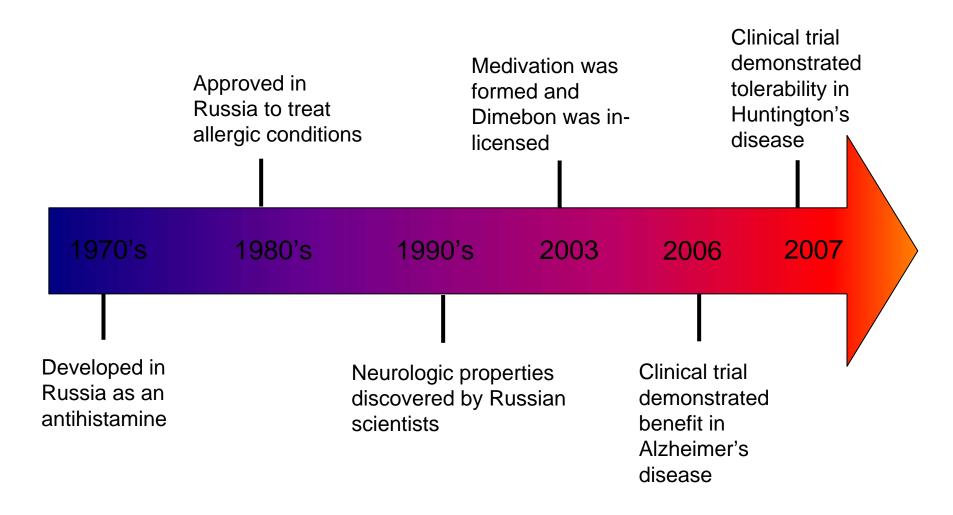
Sarah Noonberg M.D., Ph.D. Director, Clinical Development

#### Medivation, Inc.

- Publicly-traded biopharmaceutical company
- Founded in 2003
- Headquarters in San Francisco
  - Offices in Chile and India
- Mission is to efficiently develop innovative therapies for serious diseases with significant unmet medical need.
- Current pipeline:
  - Dimebon in Huntington's disease and Alzheimer's disease
  - MDV3100 in prostate cancer
- www.medivation.com



## **History of Dimebon**





#### **Major Activities in 2008**



# Phase 2 Trial of Dimebon in Patients with Mild-to-Moderate Huntington's Disease (DIMOND)

- Safety/tolerability over 3 months of treatment
- Effect on Huntington's disease symptoms
- Enrollment complete; initial results expected in July
- If results encouraging, phase 3 program to follow



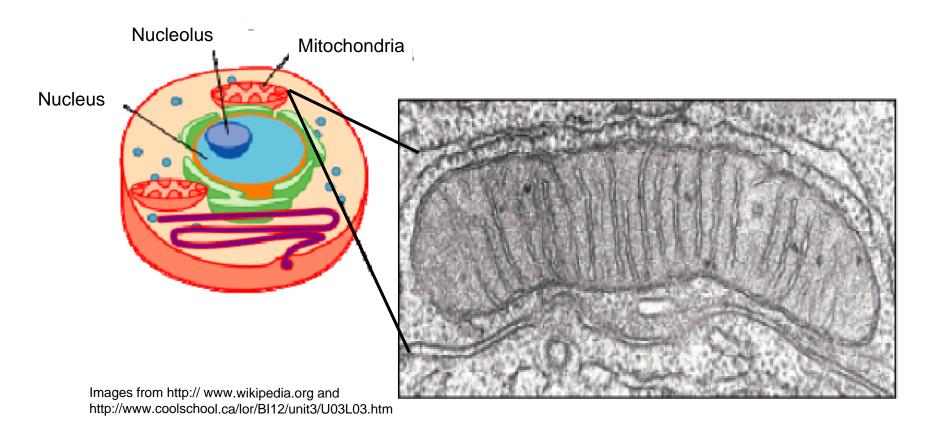
## Phase 3 Trial of Dimebon in Patients with Mild-to-Moderate Alzheimer's Disease

- Effect on Alzheimer's disease symptoms over 6 months of treatment
- Safety/tolerability
- Enrollment ongoing



#### **Dimebon Has Potent Effects on Mitochondria**

#### Human cell





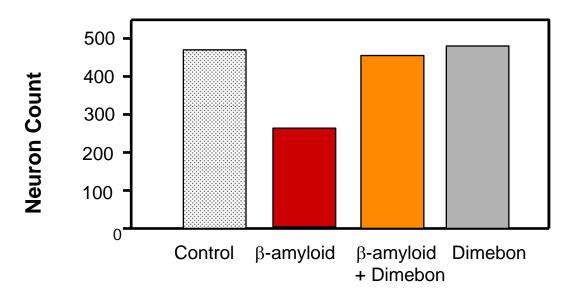
#### So...

- What are mitochondria?
  - Small structures inside of cells that turn nutrients from food into fuel
  - Tiny "gas" stations that provide fuel to cells for normal function
- What do mitochondria have to do with Huntington's disease?
  - Genetic defect is believed to interfere with normal mitochondrial function
  - Brain cells may not have enough energy to function properly
- How might Dimebon benefit Huntington's disease patients?
  - Dimebon is believed to improve mitochondrial function
  - Improved mitochondrial function may translate into healthier brain cells and improved symptoms



## Important Laboratory Effects of Dimebon

- Dimebon improves neuron survival under experimental conditions
  - Ionomycin (neurotoxin)
  - Low serum conditions (cellular stress)
  - β-amyloid (Alzheimer's disease)

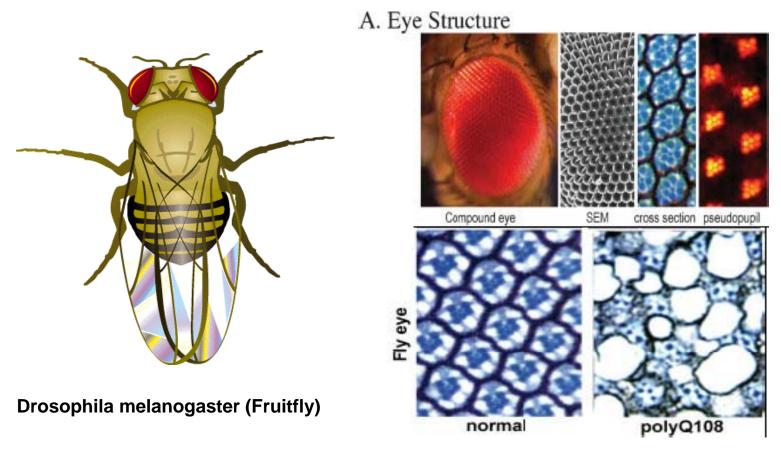


Ann NY Acad Sci, 939: 425-35, 2001



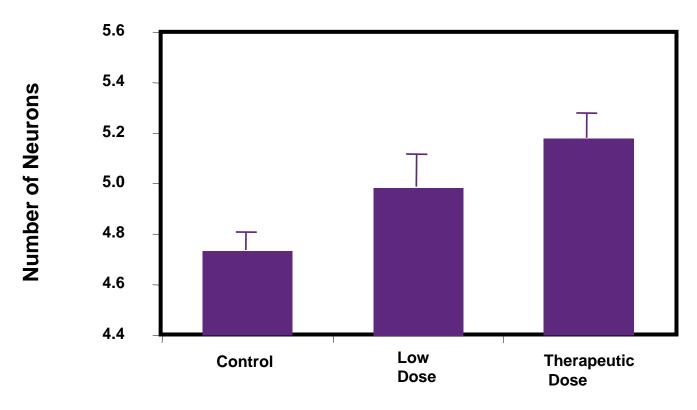
## The Huntington's Disease Fly Model

Dimebon improves neuron survival in a fly model of Huntington's disease





## **Dimebon Protected Photoreceptor Neurons**



**Dimebon Concentration in Food** 



## **Important Clinical Effects of Dimebon**

#### • Huntington's disease:

- Dimebon was generally well tolerated over 8 days of treatment
- Results supported moving forward to the current Phase 2 study

#### • Alzheimer's disease:

- Dimebon improved thinking, behavior, and overall function after one year of treatment
- General signs and symptoms were improved or stabilized in ~70% of patients
- Benefits were seen as early as 3 months and were stable or increased over time
- Dimebon was generally well tolerated
- Results supported moving forward to the current confirmatory Phase 3 study



# Similarities Between Alzheimer's Disease and Huntington's Disease

- Both diseases have similar progressive symptoms
  - Difficulty in thinking, changes in behavior, loss of independence
- Both diseases involve the accumulation of abnormal proteins in brain cells
  - β-amyloid vs. huntingtin protein
- Both diseases involve abnormalities in mitochondria function contributing to brain cell death
- Our hypothesis: Benefits demonstrated in Alzheimer's disease will translate into benefits in Huntington's disease



## Looking Ahead in our HD Program

- In the coming days:
  - Completing dosing in the DIMOND study
- In the coming weeks:
  - Analyzing initial safety and efficacy data from the DIMOND study
  - Announcing initial results (expected in July)
- In the coming months:
  - Full analysis of the DIMOND study
  - If warranted, progressing to Phase 3 studies with Dimebon



### Lastly....

**Patients** 

**Physicians** 

## Thank you

Caregivers

Study Personnel



