

# Protein Misfolding Diseases: Disruptive Technologies

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**Brain  
Research  
Centre**



**Vancouver  
CoastalHealth  
Research Institute**  
*Healthier lives through discovery*

 **PrioNet Canada**

VGH&UBC  
HOSPITAL  
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Advancing world-class health care  
for people in British Columbia



# Industrial Engagement

## **Caprion Pharmaceuticals**

Founder and Scientific Advisor  
(YYR PrP<sup>Sc</sup> Epitope)

## **Amorfix Life Sciences**

Founder and CSO, Chair BoD  
(Epitope Protection, SOD1 Epitopes, ProMIS)

## **Biogen-Idec Corporation**

(SOD1 DSE Immunotherapy)

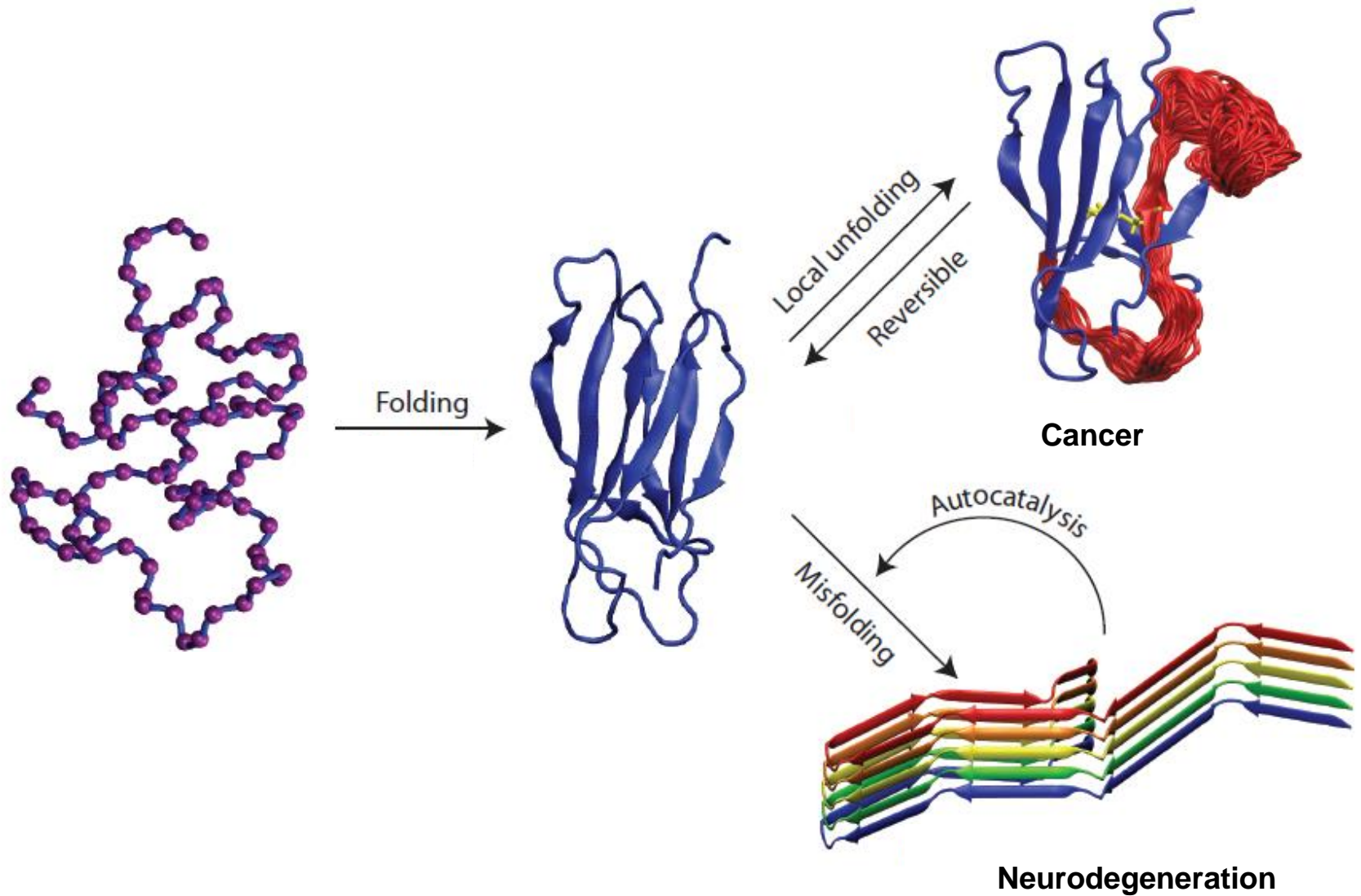
## **Cangene Corporation**

(A $\beta$  Oligomer Epitope)

## **Prothena Biosciences**

(Scientific Advisory Board)

# Disruptive Idea 1: Protein Misfolding and Disease



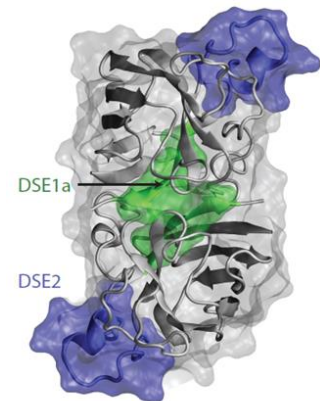
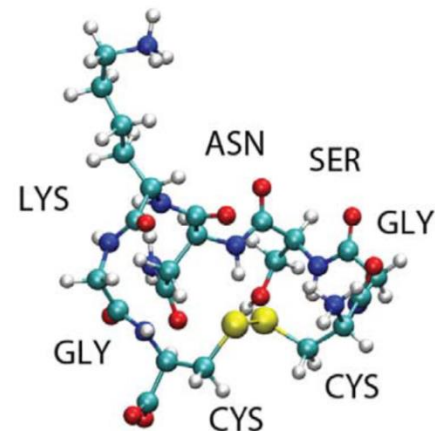
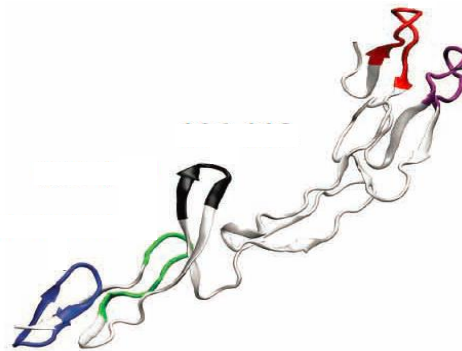
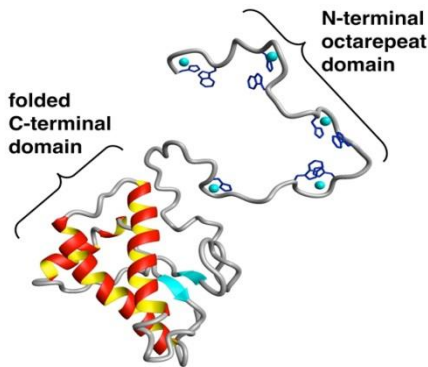
# Disruptive Idea 2: Antibodies Can Selectively Target Misfolded Proteins while Sparing Native Isoforms

**Efficacy:** Specific targeting of a pathogenic species

- Neutralization of toxicity
- Blockade of propagation
- Acceleration of degradation
- Minimal “target distraction”

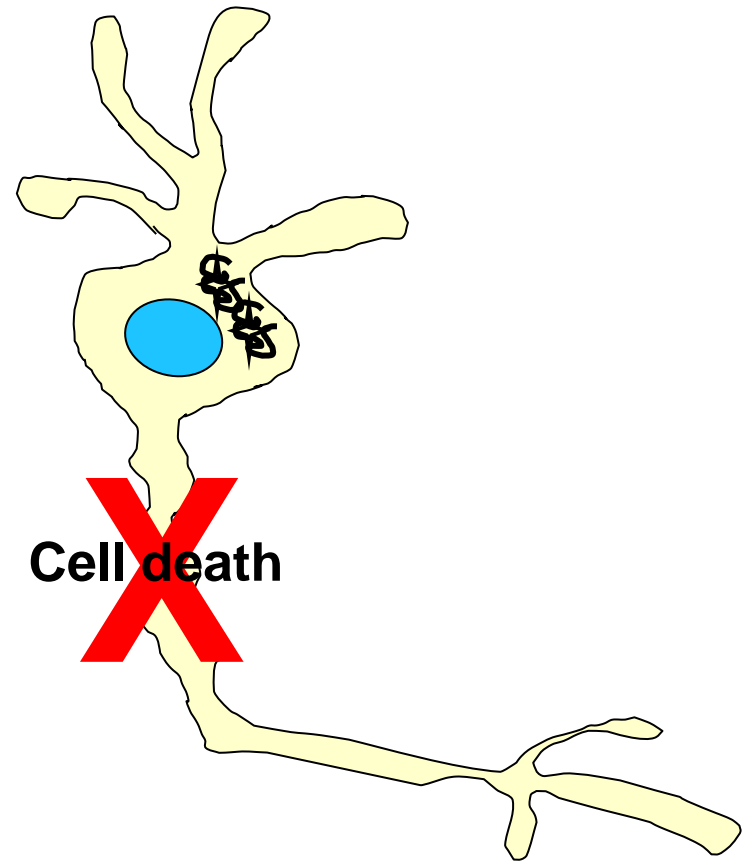
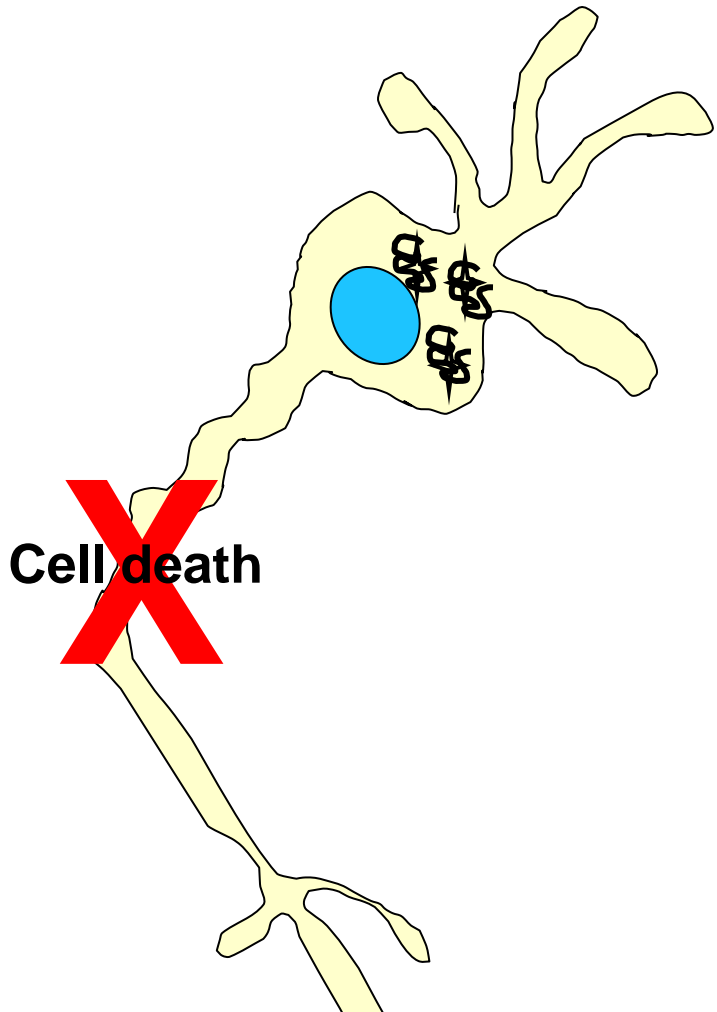
**Safety:** Selective sparing of normal proteins

- Preservation of normal function
- Minimization of autoimmunity
- Minimal regimens in therapeutic vaccines



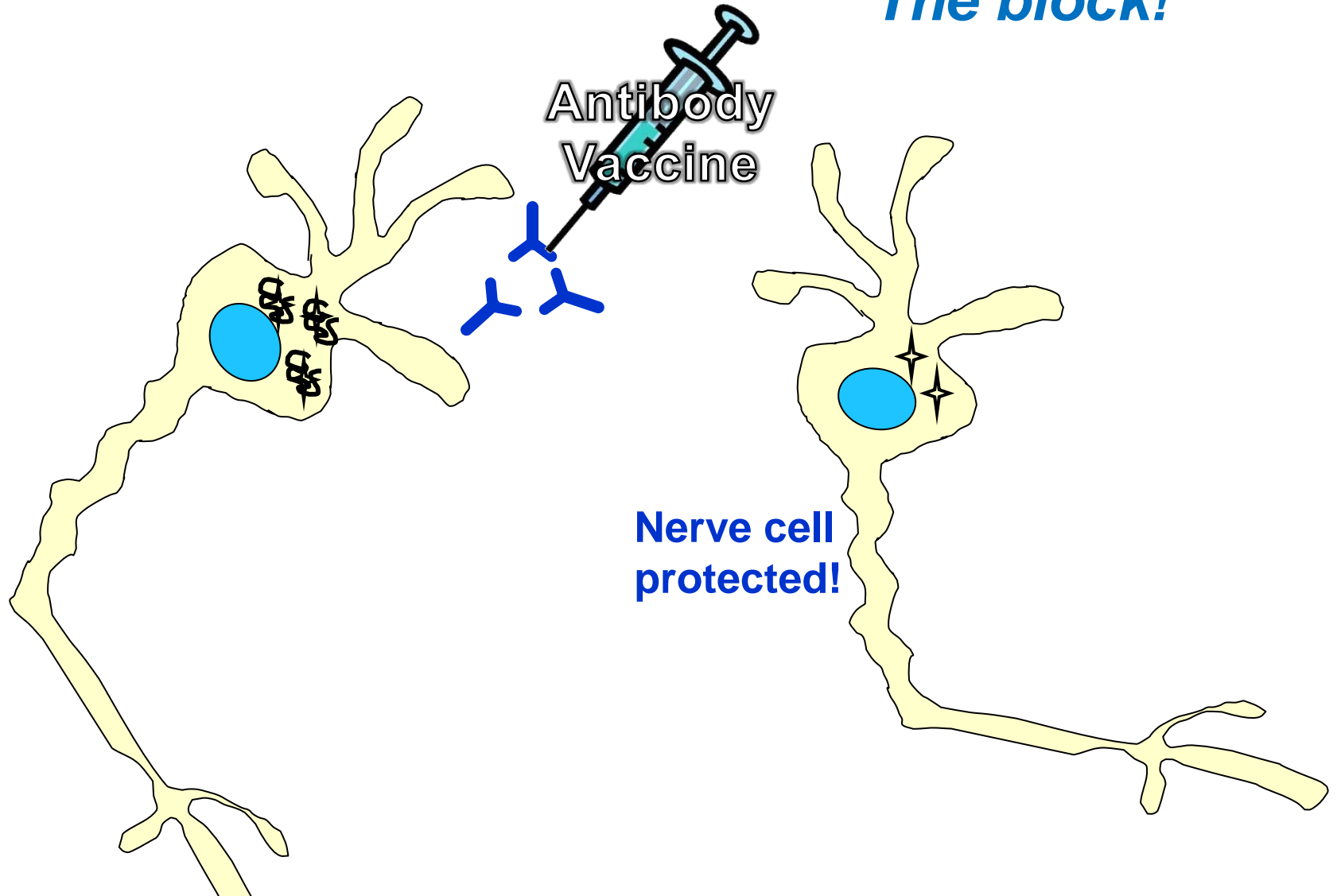
# Propagated Protein Misfolding: Mechanism

*The spread...*



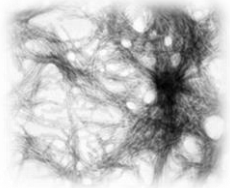
# Propagated Protein Misfolding: Treatment

*The block!*

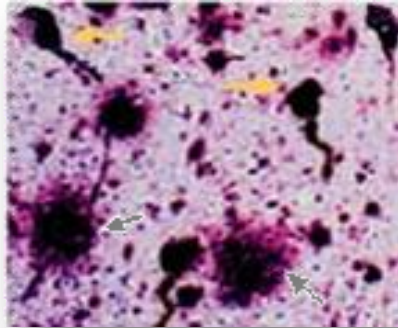


# Propagated Protein Misfolding Diseases

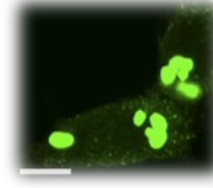
**Huntington's disease**  
(aggregates)



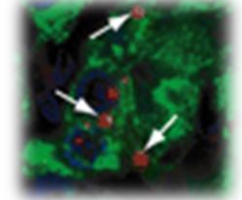
**Alzheimer's diseases**  
(plaques and tangles)



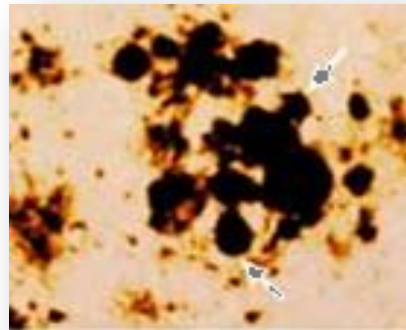
**Schizophrenia**  
(aggregates)



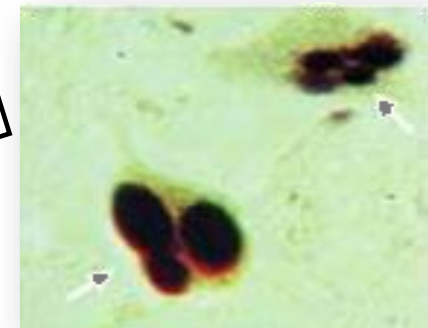
**Type 2 diabetes**  
(aggregates)



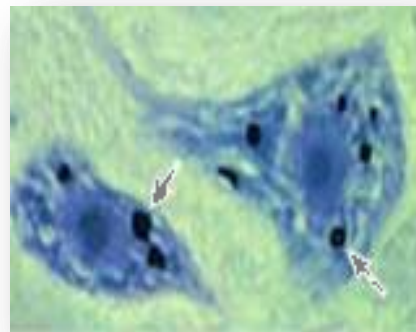
**TTR amyloid neuropathy**  
(plaques)



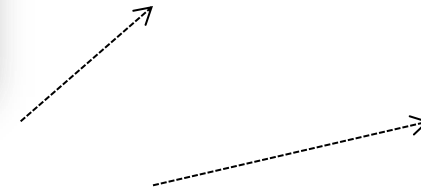
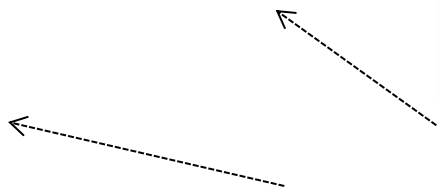
**Prion diseases**  
(PrP amyloid plaques)



**Parkinson's diseases**  
(Lewy Bodies)

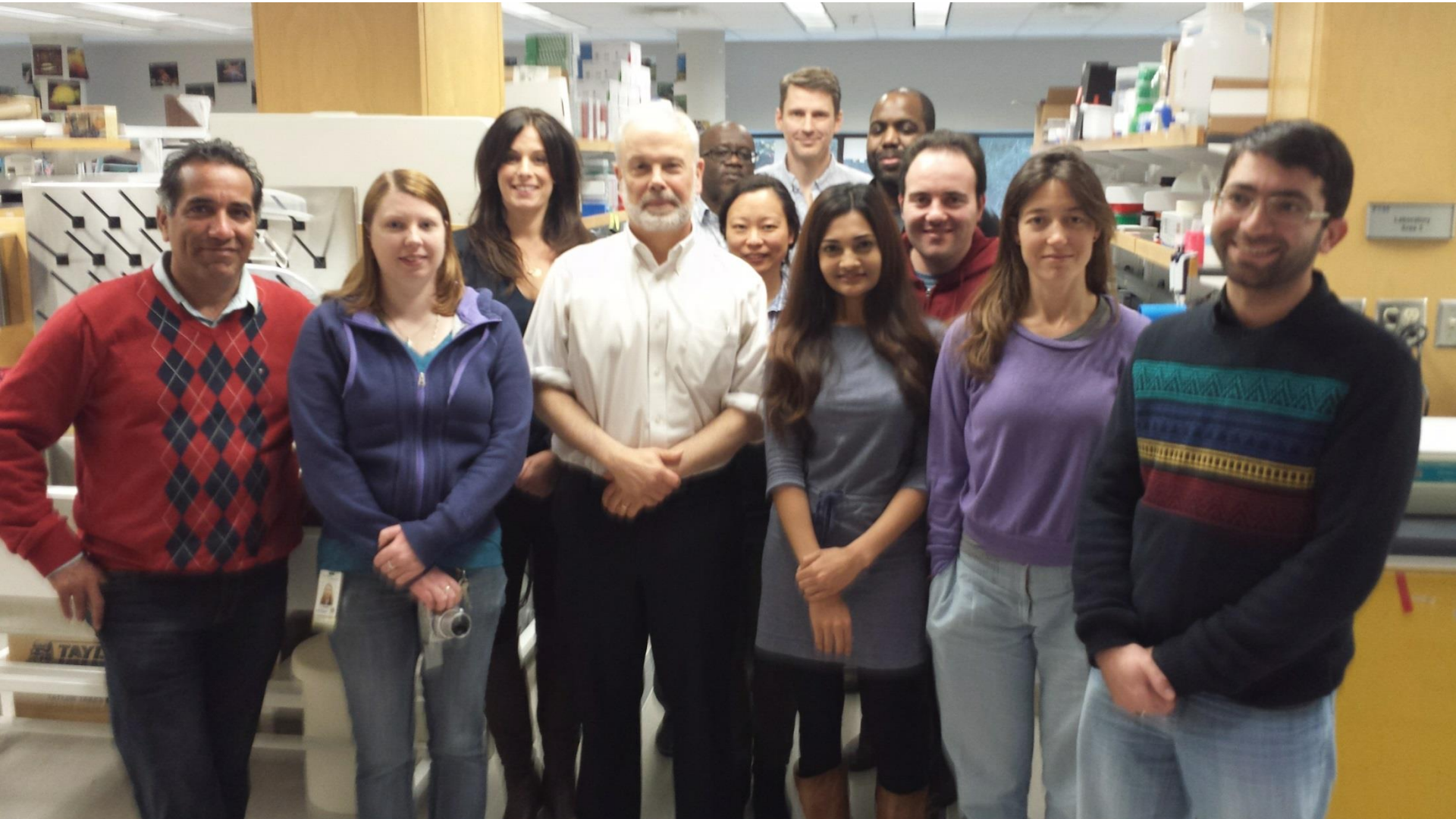


**ALS**  
(aggregates)





# Cashman Lab Brain Research Centre





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Pai group  
Prosser group

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Napper group

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Amorfix Life Sciences