R&D Revitalization

- Significant restructuring of R&D underway
  - Eliminated cardiology and oncology
  - Terminated 17 pipeline programs
  - Recruited key talent to strengthen R&D
  - Focused neurology, immunology, hemophilia
  - Selective in-licensing
- Focus in 2012 on rebuilding research
  - Discovery and Validation of new targets
  - Academic collaborations

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Building a World Class R&D Team

DOUGLAS E. WILLIAMS, PhD
EVP, Research & Development

ALFRED SANDROCK, MD, PhD
SVP, Development Sciences & Chief Medical Officer

GLENN PIERCE, MD, PhD
SVP, Global Medical Affairs & Hemophilia

TIMOTHY HARRIS, PhD, DSC
SVP, Translational Medicine

JO VINEY, PhD
VP, Immunology Research

SPYROS ARTAVANIS-TSAKONAS, PhD
SVP, Chief Scientific Officer

TERESA COMPTON, PhD
VP, Research

THERESA PODREBARAC, MD
VP, Medical Research, Immunology

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Pipeline Early 2011

	PHASE I	PHASE II	PHASE III	FILED
BG-12	Multiple Sclerosis			
PEGylated-IFNβ1a	Multiple Sclerosis			
rFIXFc	Hemophilia B			
rFVIIIFc	Hemophilia A			
Daclizumab	Multiple Sclerosis			
GA101	Chronic Lymphocytic Leukemia & Non-Hodgkin's Lymphoma			
Dexpramipexole	Amyotrophic Lateral Sclerosis			
Anti-TWEAK	Lupus Nephritis			
Anti-LINGO	Multiple Sclerosis			
Anti-CD40 Ligand	General Lupus			
Neublastin	Neuropathic Pain			
BIIB037	Alzheimer's Disease			

KEY

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Pipeline Today

	PHASE I	PHASE II	PHASE III	FILED
BG-12	Multiple Sclerosis			
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TYSABRI	SPMS			
Anti-TWEAK	Lupus Nephritis & RA			
ACQUIRED, FEB 2012 STX-100	Idiopathic Pulmonary Fibrosis			
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COLLABORATION, OCT 2011 Syk Inhibitor	RA, SLE, & Atopy			
Anti-CD40 Ligand	General Lupus			
Neublastin	Neuropathic Pain			
BIIB037	Alzheimer's Disease			
COLLABORATION, JAN 2012 ISIS-SMNRx*	Spinal Muscular Atrophy			

KEY

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*ANTISENSE THERAPY: GENETICALLY TARGETED THERAPY

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Early Stage Pipeline

ANTI-LINGO

Multiple Sclerosis

- Expect to initiate Phase II trial in 2H 2012

NEUBLASTIN

Neuropathic Pain

- Currently in Phase I

BIIB037

Alzheimer's Disease

- Currently in Phase I

ISIS-SMNRX

Spinal Muscular Atrophy

- Currently in Phase I

ANTI-TWEAK

Lupus Nephritis

- Expect to initiate Phase IIb trial in 2H 2012

ANTI-CD40 LIGAND

General Lupus

- Currently in Phase I

SYK INHIBITOR

RA and SLE

- Expect to initiate Phase II trial in 2H 2012

STX-100

Idiopathic Pulmonary Fibrosis

- Expect to initiate Phase II in 2H 2012

IMMUNOLOGY NEUROLOGY

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R&D Vision

Create the Best Science Driven R&D Pipeline

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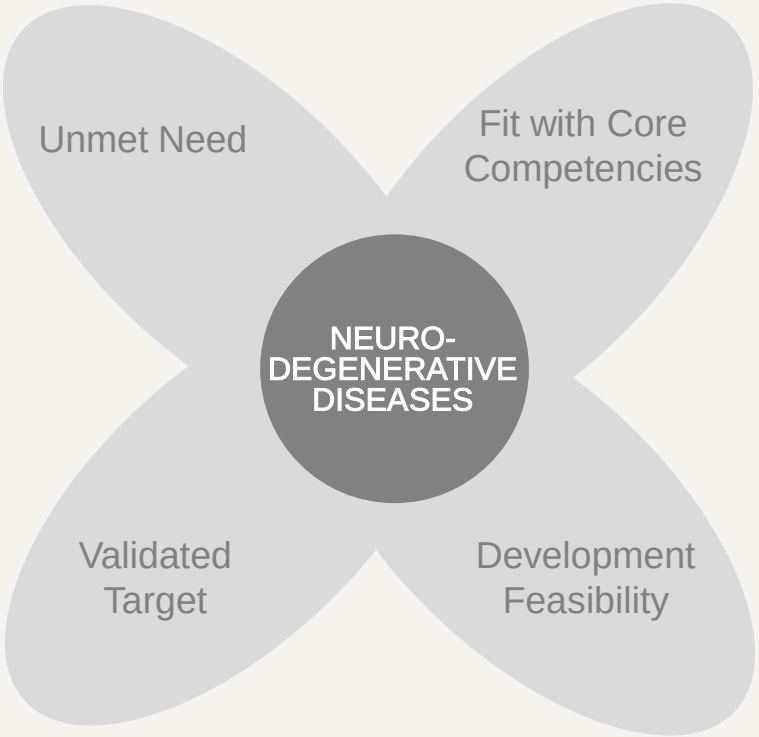
Neurology Discovery Strategy and Focus

ALIGNMENT WITH NEUROLOGY R&D MISSION AND CORE STRATEGY



PROTECTION AND REPAIR

Structural Repair – myelin

Cytoprotection – oxidative/metabolic stress

PROTEINOPATHY

Disease progression - immunotherapy

Protein misfolding - autophagy

GENETICALLY WELL-DEFINED DISEASES

TRANSLATIONAL NEUROLOGY

Neuroimaging – MRI, PET, SPECT

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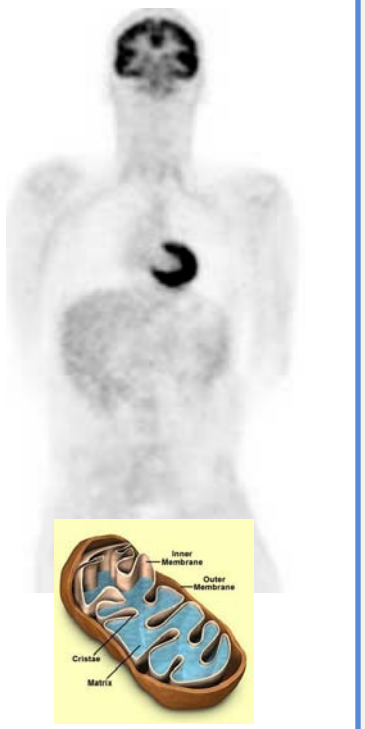
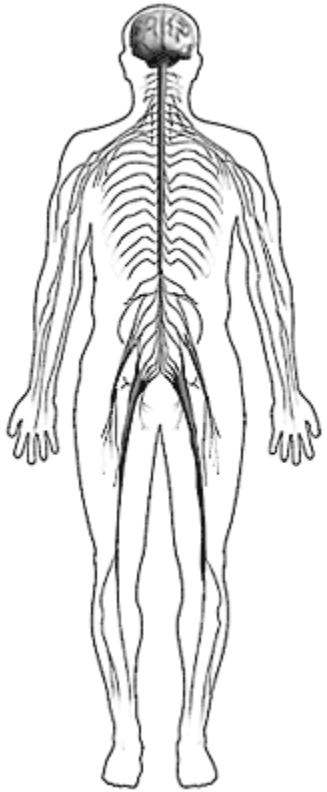
The Achilles Heel of the Nervous System: Enhanced requirements for…

Metabolic integrity



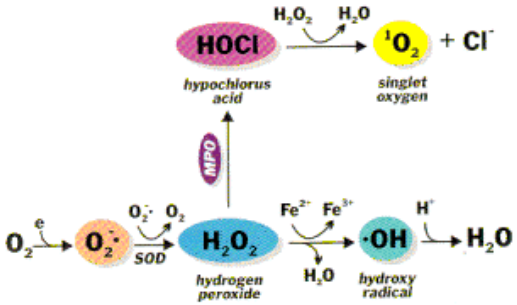
Dexpramipexole

Structural Integrity
Glial Support



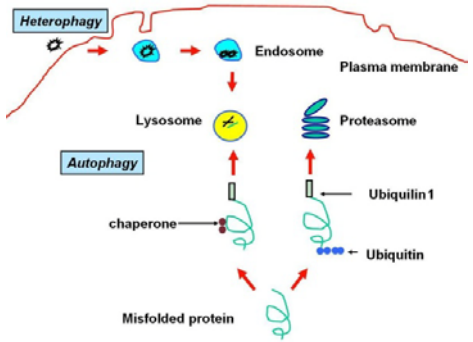
Lingo, neublastin

Oxidative Integrity



BG12

Macromolecular Integrity



Anti-proteinopathy mABs

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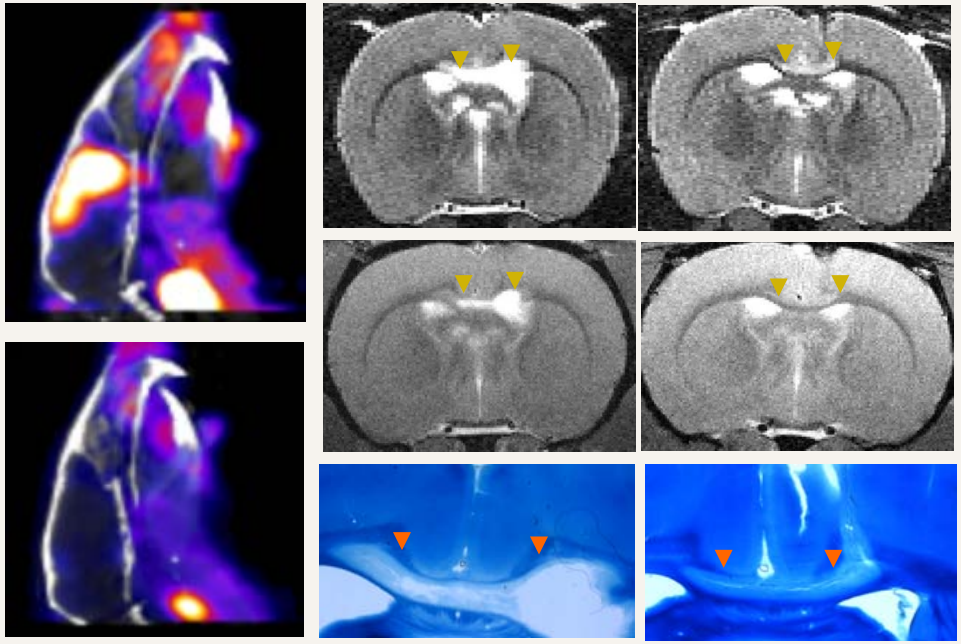
Focus Area: Translational Neuroscience



Discovery Translational Imaging Approaches:

PET/SPECT/CT imaging

Small animal MRI

- Technology advances in small animal imaging allow incorporation of clinical measures into preclinical animal studies
- Endpoints allow direct translation of PK/PD, biomarker and efficacy endpoints from animals to humans

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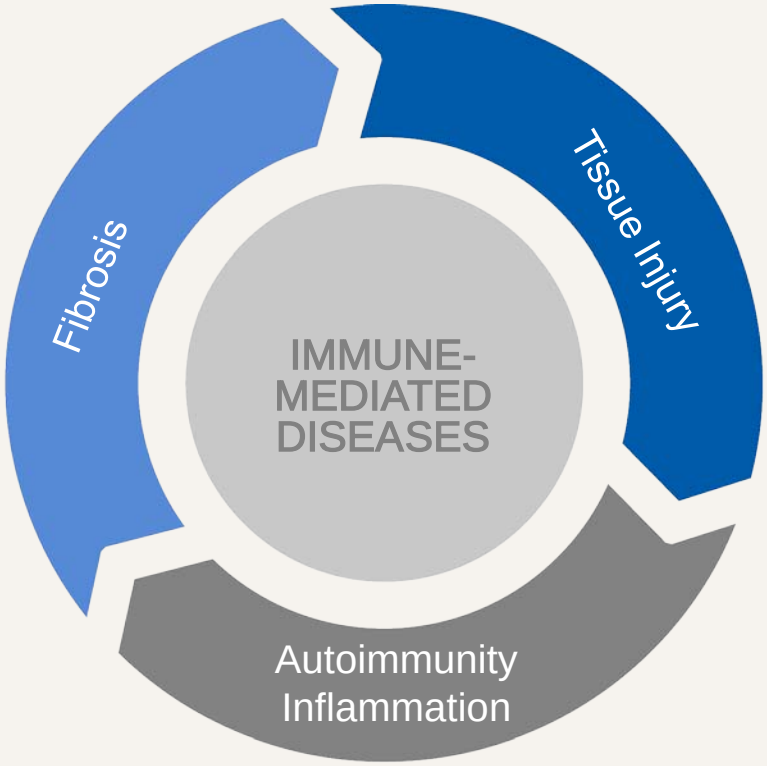
Immunology Therapeutic Area Strategy

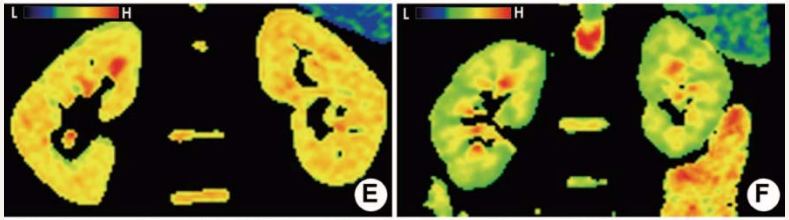
Develop treatments for underserved immune-mediated diseases in the areas of fibrosis, autoimmunity and tissue injury



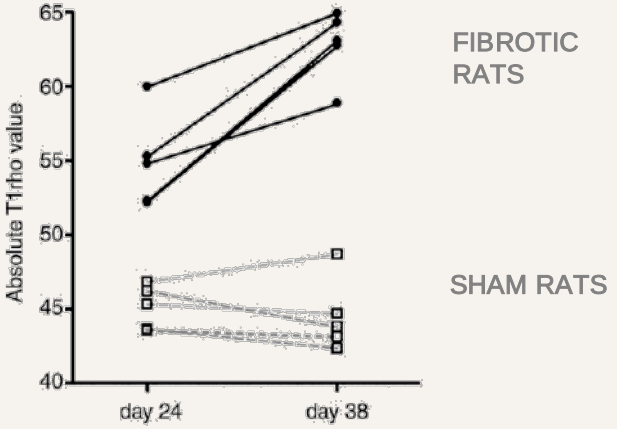
Functional MRI is being Developed as a non-invasive tool to follow Renal Injury and Scar

- Phase 0 study with the objective of developing a non-invasive endpoint that could be used in lupus nephritis and other renal diseases
- Exploring whether renal functional MRI techniques discriminate between renal damage and inflammatory activity in lupus nephritis
- Collaboration with UCLA, Emory and Ohio State University

ADC map (DW-MRI)



Healthy kidney Chronic glomerulonephritis



T1ρ in animal models of liver fibrosis

Revitalizing Discovery Research

- De-emphasized target identification and validation in recent years
- Re-emphasizing importance to BIIB pipeline and future
  - Proprietary targets and first in class drug candidates
  - Discovery culture enhances scientific quality
- Re-allocation and recruiting new resources to support efforts
- Leveraging select academic partnerships in the short term

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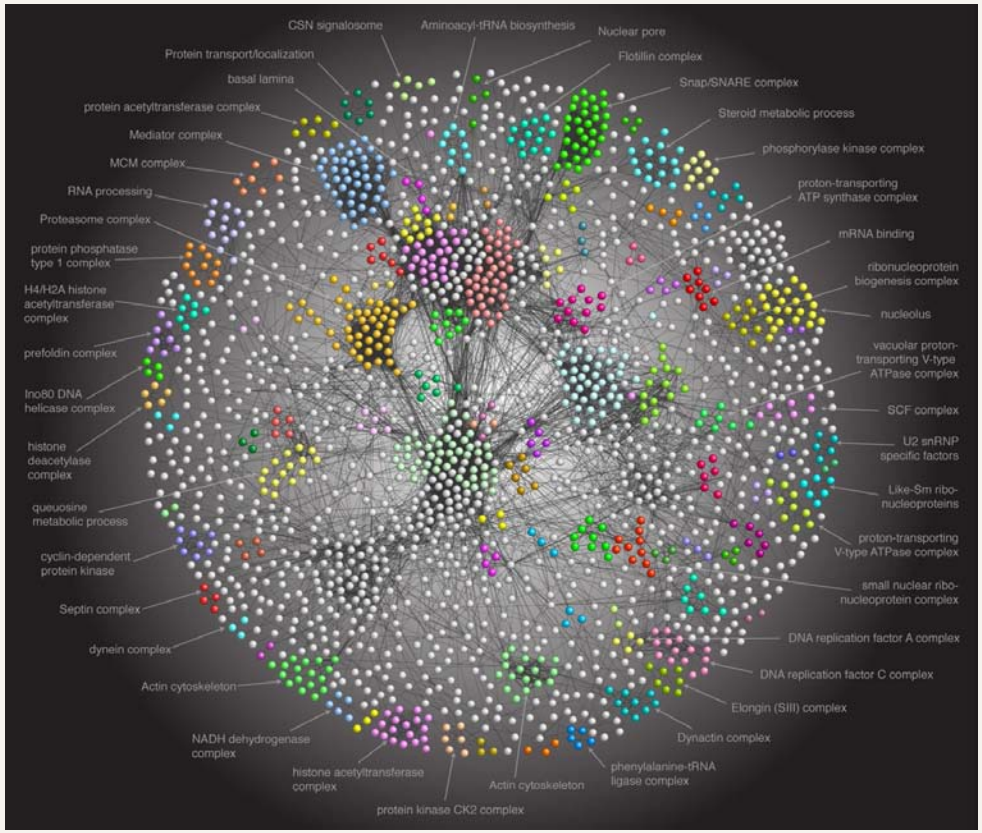
Harvard Interactome Project

- Builds on successful Drosophila program
- Plan to assess the human proteome over 3-4 years
- Allows an understanding of what proteins interact with other proteins
- 200 proprietary proteins to initiate the study
- Secondary screens will define novel drug targets and pathways
  - Cell type specific screens
  - Drug perturbation screens
  - Mutant protein screens



DROSOPHILA INTERACTOME COURTESY OF SPYROS ARTAVANIS-TSAKONAS, PhD

ALS Consortium

- Consortium involving Rick Myers (Hudson Alpha), David Goldstein/ Bob Brown (Duke and U Mass) and Tom Maniatis (Columbia)
- Plan to sequence whole genome and exomes from 600 ALS patients including some from ALS families over next three years
- Complement with cell biology approaches
  - Mitochondrial function
  - Axon repair
  - Synaptic vesicle biology
- Information will be used to:
  - Identify new ALS genes that could be used for patient stratification
  - Find new pathways for validation as drug targets

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Late Stage Pipeline Events

PROGRAM	PHASE	INDICATION	EXPECTED MILESTONE
BG-12	Phase III	RRMS	Under Active Review with FDA & EMA
Long-Lasting Factor IX	Phase III	Hemophilia B	Data readout 2H 2012
Long-Lasting Factor VIII	Phase III	Hemophilia A	Data readout 2H 2012
Dexpramipexole	Phase III	ALS	Data readout 4Q 2012
PEGylated Interferon	Phase III	RRMS	Data readout 1H 2013
GA101 (RG7159)	Phase III	CLL, NHL & DLBCL	Data readout for CLL in 2013
Daclizumab-HYP	Phase III	RRMS	Data readout 2014
TYSABRI	Phase III	SPMS	Data readout in 2015

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