Session III: Gene-Targeting Therapy Technologies for CNS Disorders

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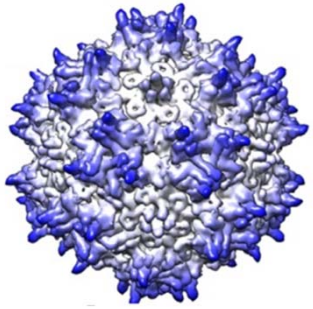
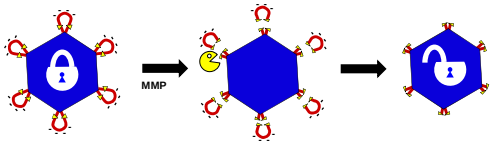
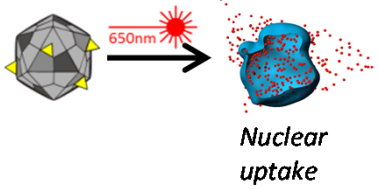
Synthetic Virology

INPUT →  → OUTPUT

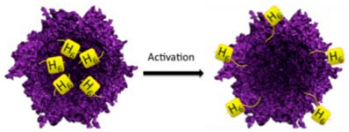
Provector



Lumivector



Activatable Peptide Display



Call for more

engineers,

computational biologists,

biophysicists,

etc.

to work in gene therapy

Acknowledgments

- Caleb Bashor (Rice)
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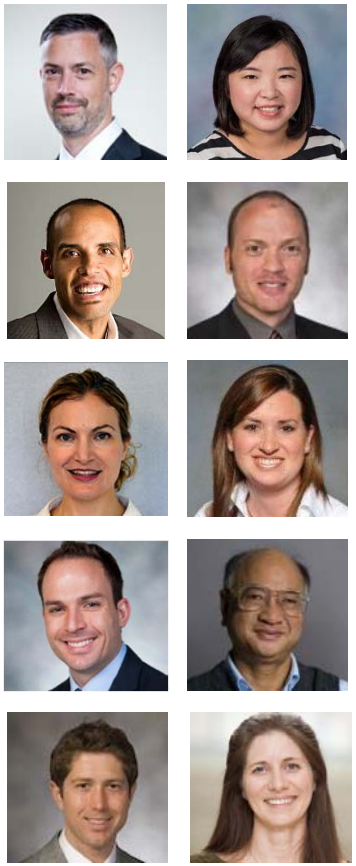
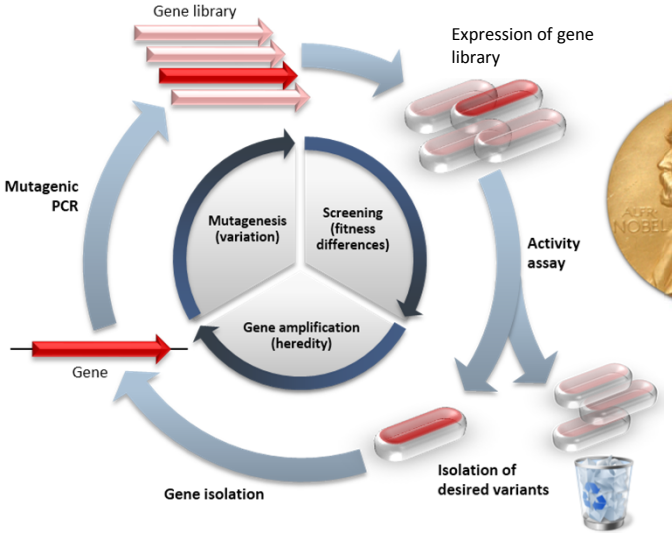
- Jacob Robinson (Rice)
- Ka-Yiu San (Rice)
- Jeff Tabor (Rice)
- Danielle Tullman-Ercek (Northwestern)



Delivery Vector | Protein Engineering

Bioengineering Chemical Engineering Physical Biology

Directed Evolution



Frances Arnold

Greg Winter

Enzymes

Laundry detergents – lipases
Pesticides – P450s
Biofuels – cellulases
Antibacterials - lysozyme

Antibodies

Anthrax treatment - ABthrax
Cancer drugs – Portrazza
Macular degeneration – Lucentis
Arthritis – Humira

Develop better delivery vectors

(Image adapted from Thomas Shaftee, U Cambridge)

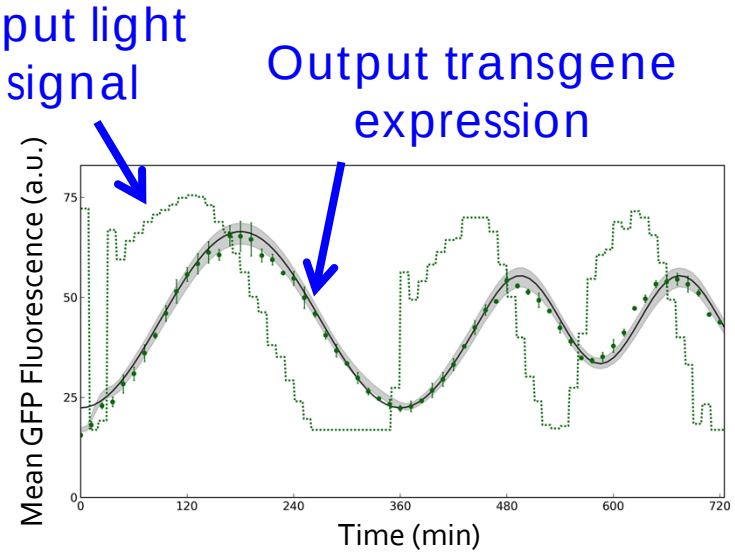
(Slide courtesy of Danielle Tullman-Ercek)

Viviana Gradinaru will discuss more

Cargo Expression | Synthetic Biology

Bioengineering Chemical Engineering
Electrical Engineering Mechanical Engineering

Example: Optogenetic Transcriptional Control of Gene Expression



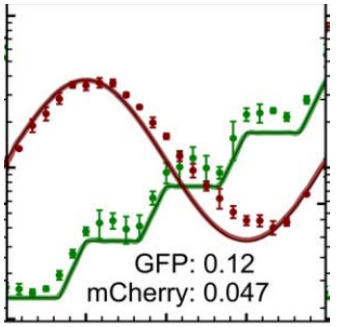
(Olson et al., Nat Methods 2014)

(Slide courtesy of Jeff Tabor)

Control Systems Engineering

- Input, output, process, sensor, feedback → control

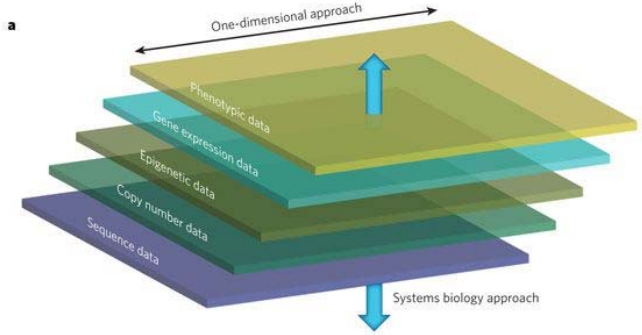
Multiplexed Control

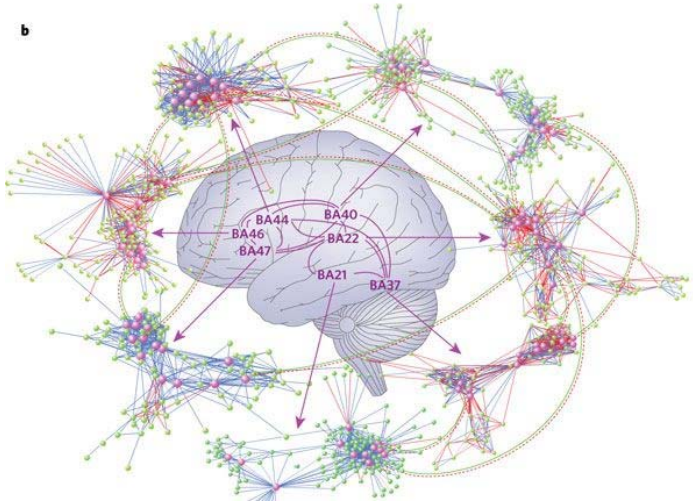


Control transgene expression profiles in complex environments

Cargo Choice | Systems Biology

Bioengineering Computer Science Statistics





Phenotypic
Gene Expr
Epigenetic
Copy #
Sequence

- aka Computational Neuroscience
- Data science
- Extract non-obvious patterns from complex datasets
- Develop multi-pronged therapeutic approaches

Treat more complex diseases

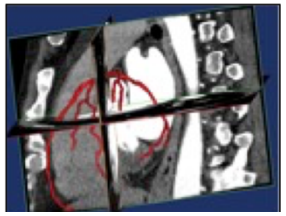
(Geschwind and Konopka, Nature 2009)

Administration | Computational Fluid Dynamics

Aerospace Engineering Bioengineering
Chemical Engineering Mechanical Engineering

Quantitative modeling of transport in complex environments

Patient-Derived Imaging Data

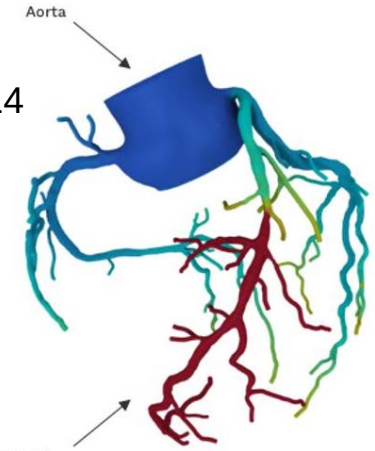


Data Segmentation

Patient-Specific 3D Geometries

Heart Flow

FDA approved 2014



(Forbes.com)

Computational Modeling

Develop better administration strategies

(Slide courtesy of Amanda Randles)

Orchestrate Coherent Endeavor

Structure

decoupled, organized,
and collaborative

Connectors

draw lines, be translators,
& facilitate synthesis