**Gene Therapy in Alzheimer's Disease: Novel Therapies and Ethical Aspects of Somatic Gene Editing (2020-2021)**

Goals and Objectives:

Gaining insight into the molecular and biological pathways of Alzheimer’s disease, while exploring the ethical, legal, and social implications of a gene-therapy for Alzheimer’s. Creation of an open line of communication between team members with constructive feedback at regular intervals in order to best advance the overall interdisciplinary goal of the project.

Team Goals:

1. Each team member has a basic understanding of the research being conducted by other members
2. Regular team meetings
3. Team members are able to both supply effective feedback during team progress meetings and respond to the suggestions offered by other team members

Individual Goals:

1. Ethics: Utilizing literature for the purpose of further exploration of the implications of a future gene therapy; project management; publications regarding the challenges associated with a Gene Modifying Therapy (GMT) with consideration given towards (the healthcare system, infrastructure, and payment models)
2. Viral Vector Core: To design an allele-specific CRISPR/Cas9 -based lentivirus to downregulate APOE4 and rescue disease-related phenotypes in LOAD
3. Chiba-Falek Lab: To successfully develop a human-based model system in cell culture to investigate the molecular pathology of late onset Alzheimer’s disease and to screen for the effectiveness of potential gene therapies targeted the risk-allele APOE4; to characterize AD and age-related phenotypes in APOE 2/2, 3/3, 4/4 hiPSC-differentiated neurons, astrocytes, and microglial cells.

Milestones and Timelines

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| **Month 1** | **Monthly milestones**  **●** To understand overall team objectives, meet with mentor, familiarize yourself with workspace |
| **Month 2** | **Monthly milestones**  **●** Learn basic skills associated with research role, become familiar with background literature |
| **Month 3** | **Monthly milestones**  **●** Begin shadowing (if applicable), discover personal goals for research |
| **Month 4** | **Monthly milestones**  **●** Continue shadowing, work on personal projects as assigned by mentor, communicate with other team members, offer updates (first presentation) |
| **Month 5** | **Monthly milestones**  **●** Become more comfortable with independence in the workspace, report to mentor at regular intervals about progress |
| **Month 6** | **Monthly milestones**  **●** Reflect on work completed in the summer  ●  Team bonding outing |
| **Month 7** | **Monthly milestones**  **●** Revise personal plan about goals for the academic semester  ●  Lay out independent project expectations and goals |
| **Month 8** | **Monthly milestones**   * Begin working on the personal project (lab or publication) |
| **Month 9** | **Monthly milestones**   * Attend and participate in a poster session |
| **Month 10** | **Monthly milestones**   * Continue working on personal project and begin work on a final presentation |
| **Month 11** | **Monthly milestones**   * Present final presentation |
| **Month 12** | **Monthly milestones**   * Attend a national meeting relating to Neuroscience, Alzheimer’s Disease, and/or Gene Therapy |

Roles

Mentors: Oversee student projects and lend expertise and provide support as necessary, assign readings for students to keep informed

Students: Meet with mentor on a regular basis, individual project management, weekly progress update, communicate ideas, concerns, and issues with mentor and team members, continue to develop applicable research skills

Processes

* Monthly Bass team meetings
* Interval one-on-one meetings with team leader
* Share relevant data and information through email correspondence
* Decisions made on a consensus-basis with the guidance of team leaders
* Team will track progress through presentations and updates during monthly team meetings

Team Norms: See Attached File

Signatures: