

GTSAB REPORT

Recombinant DNA Advisory Committee

June 9, 2015



National Institutes
of Health

Protocols Submitted for Second Quarter 2015

40 total submissions

- **Two were selected for public review**

Protocols not Selected this Quarter

Diseases		
26 Oncology	1 Heart failure	1 Arthritis
6 Monogenic diseases	1 Brain injury	1 ALS
2 Infectious diseases		

Vectors		
8 Retroviruses	9 Plasmids	4 Adenoviruses
7 Lentiviruses	5 AAV	2 attenuated <i>Listeria monocytogenes</i>
2 RNA	1 HSV	

Serious Adverse Events

21 serious adverse events were reviewed by the GTSAB from 14 protocols, including initial and follow-up reports.

Opening of New Protocols Second Quarter 2015

Nine protocols notified OBA of enrollment (MIC1 submission).

- **Two were publicly reviewed.**
- **One previously responded.**

Protocols Initiated this Quarter

(publicly reviewed)

1001-1024:

Lymphodepletion Plus Adoptive Cell Transfer with CXCR2 and NGFR Transduced T-Cells Followed by High Dose Interleukin-2 in Patients with Metastatic Melanoma

1007-1050:

Phase I Study of an Active Immunotherapy for Asymptomatic Phase Lymphoplasmacytic Lymphoma with DNA Vaccines Encoding Antigen-Chemokine Fusion

Protocols Initiated this Quarter

(not publicly reviewed)

- 1210- 1187:** Clinical Research Study of Autologous Bone Marrow Transplantation for Sickle Cell Disease (SCD) Using Bone Marrow CD34+ Cells Modified with the Lenti/ β AS3-FB Lentiviral Vector
- 1304-1227:** A Phase I Study of Intratumoral/peritumoral Herpes Simplex Virus-1 Mutant HSV1716 in Patients with Refractory or Recurrent High Grade Gliomas (HGG)
- 1310-1254:** A Phase 1 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin[®] BB305 Lentiviral Vector in Subjects with Severe Sickle Cell Disease
- 1310-1255:** A Phase 1 Safety and Tolerability Study in Subjects with Choroideremia Using an Adeno-Associated Virus 2 Vector to Deliver the Normal Human Choroideremia Gene [AAV2-hCHM] to the Retina
- 1407-1329:** A Phase I/IIa Study of RS1 Ocular Gene Transfer for X-linked Retinoschisis
- 1412-1366:** A Phase I/II Study Administering Peripheral Blood Lymphocytes Transduced with a Murine T-Cell Receptor Recognizing Human Thyroglobulin to Patients with Thyroglobulin Expressing Thyroid Cancer
- 1501-1367:** Double-Blind, Placebo Controlled Phase III Trial of Maintenance FANG[™] (bi-shRNAfurin and GMCSF Augmented Autologous Tumor Cell Immunotherapy) for High Risk Stage III/IV Ovarian Cancer

Upcoming Event

June 10, 2015

Workshop:

Cytokine Release Syndrome after T-Cell Immunotherapy

Outcome Goals:

**Establishing Definitions, Developing Criteria, and
Optimizing Management**

Location:

**Natcher Conference Center (NIH Campus)
Building 45, Conference Room E1/E2
Bethesda, MD**

Webcast Address:

www.videocast.nih.gov