

GTSAB REPORT

Recombinant DNA Advisory Committee

June 9, 2011



Protocols Submitted for 2nd Quarter 2011

- ▶ 19 submissions total
 - 11 Protocols Not Selected:
 - 7 for cancer
 - 1 for heart disease
 - 1 for coronary artery disease
 - 1 for retinitis pigmentosa
 - 1 for Fanconi anemia

Vectors:

- 4 plasmid
- 2 adenovirus
- 2 retrovirus
- 1 genomic DNA (no vector)
- 2 lentivirus

MIC1 Submissions

2nd Quarter 2011

- ▶ 13 Protocols submitted MIC1s to OBA indicating enrollment
- ▶ 4 Protocols were reviewed by the RAC at public meetings
 - ▶ Please refer to the meeting materials for details of the response.

Serious Adverse Events

- ▶ 15 serious adverse events were reviewed by the GTSAB from 11 protocols, including initial and follow-up reports. No reports need additional discussion at this time.

Symposium : Gene Modified T cells

June 2010

▶ Summary of Meeting Published

- Ertl, H.C.J., Zaia, J., Rosenberg, S., June, C.J., Dotti, G., Kahn, J., Cooper, L. J.N, Corrigan–Curay, J., Strome, S., *Considerations for the Clinical Application of Chimeric Antigen Receptor T Cells: Observations from a Recombinant DNA Advisory Committee Symposium Held June 15, 2010*, **Cancer Research**,71(9) 2011.
- ▶ **RAC Points to Consider Reviewed by GTSAB and will also be Posted to OBA Website**

http://oba.od.nih.gov/rdna/rdna_symposia.html

RNA Oligonucleotides

- ▶ **RAC recommended NIH\OBA convene a conference to examine what is known about RNA therapeutics from the clinical experience to date.**
 - ▶ **What are the unanswered research questions needed to develop this emerging field safely, in particular protocols using microRNAs?**
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Planning Committee

▶ **Bishow Adhikari, Ph.D.,**
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RNA Oligonucleotides: Emerging Clinical Applications

December 15–16, 2011

- ▶ **Session I- Overview of Current Clinical Approaches using RNA Oligonucleotides**
 - **Overview of the Clinical Experience with RNAi**
 - **Panel Discussion**
 - **What has been learned about biodistribution, persistence of oligos, models for off-target activity?**
 - **What are the key challenges that need to be addressed to move the field forward?**

RNA Oligonucleotides: Emerging Clinical Applications

December 15–16, 2011

- ▶ **Session II- microRNA: Basic Understanding and Potential Clinical Applications**
 - **Overview of Emerging Translational Research using miRNA**
 - **Development of Preclinical Models, Supporting Technologies, and Tools**
 - **Panel Discussion**
 - **Assays and models**
 - **Potential epigenetic effects**
 - **Key unanswered questions**
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**Update on OBA Protocol # 0707-864
An Open Label Dose-Escalation Study of a Self
Complementary Adeno-Associated Viral
Vector (scAAV2/8-LPI-hFIXco) for Gene
Therapy of Hemophilia B**

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