

# APBDRF

## Feedback: 12<sup>TH</sup> ANNUAL SCIENTIFIC ADVISORY BOARD MEETING

<p><b>How are you connected to APBDRF? (Check all that apply)</b></p>	<input type="checkbox"/> Research Scientist on APBD <input type="checkbox"/> Research Scientist on related disease/disease mechanism <input type="checkbox"/> Physician <input type="checkbox"/> Other Health profession (e.g. genetics, clinical, lab, etc.) <input type="checkbox"/> Patient/family member <input type="checkbox"/> Friend/supporter <input type="checkbox"/> APBDRF staff/adviser <input type="checkbox"/> Other: _____
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<p><b>Overall, what aspects of the conference were most interesting and valuable to you?</b></p> <input type="checkbox"/> The quality, content and findings of presentations <input type="checkbox"/> Opportunity to talk/meet directly with researchers/colleagues <input type="checkbox"/> Opportunity to learn about the range of research underway <input type="checkbox"/> Hopeful implications of the research – the potential promise for a treatment or cure <input type="checkbox"/> Other: _____
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Overall, how would you rate the conference?						
Poor		Ok			Excellent	
1	2	3	4	5	6	7

Over →i

**Which of the sessions (presenter/moderator) did you attend? (check those you attended)**

**Monday**

- 1. GBE Stabilization (Various presenters)
- 2. Genetic Interventions (Various)
- 3. MyJewishGenetics (Schreiber-Agus)
- 4. Duke GSD IV (Kishnani, Sun)
- 5. Triheptanoin (Schiffmann)
- 6. Proposed guaiacol trial (Kakhlon)
- 7. Clinical Endpoint discussion (various)
- 8. High Throughput Screening (Kakhlon)
- 9. Life Through a Lens (Screening, Zuckerman)

**Tuesday**

- 10. Repurposed Drug Initiatives (Bloom, various) 
  - a. Guaiacol (Kakhlon)
  - b. Recombinant Glycogen Synthase (Kakhlon/Yue)
  - c. Ibudilast/gualfenesin (Akman)
  - d. Pharmacophore-based small molecules (Kakhlon)
- 11. Genetic Testing, Natural History Studies, Biobanks
  - a. (Correia)
  - b. Patient Registry (Schiffmann)
  - c. Genetic Testing (Kornreich)
  - d. Carrier screening advances (Cecchi)
  - e. Natural History Studies, NORD (Rossov)
  - f. Orphan Products-Natural Hist. (Jones)
  - g. Biobank of fibroblasts (Kakhlon)

Which of the above sessions did you find most interesting/informative? Most understandable, clearest? Most promising to pursue further? Please list top choices in each category, by number and letter (from above listing):

	<u>Informative/interesting</u>	<u>Clear/understandable</u>	<u>Promising</u>
1.	_____	_____	_____
2.	_____	_____	_____
3.	_____	_____	_____
4.	_____	_____	_____

Do any of the treatment approaches presented stand out as most urgent for the APBDRF to press forward with support, and if so, which one? \_\_\_\_\_

What improvements/changes would you recommend for future conferences (other than A/V technology?) Please provide us your candid comments or recommendations.

- Thank you -