4th Drug Discovery & Development Symposium for Pulmonary Hypertension 10-11 July 2017

Feedback analysis

Overall, the symposium was well received, with delegates rating it 'Excellent' with 71.29%; 'Good' with 28.11% and 'Poor' at 0.60%.

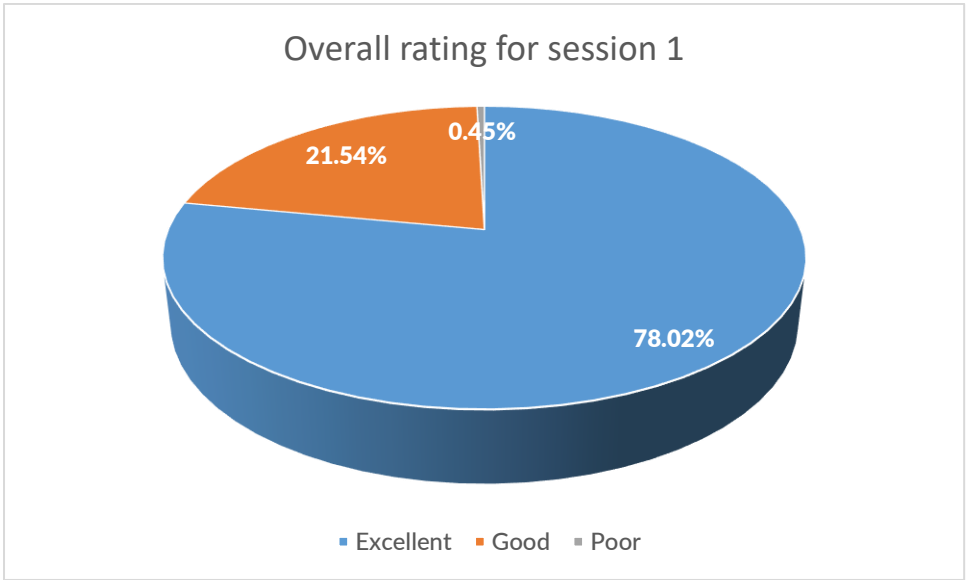
The top three rated talks were:

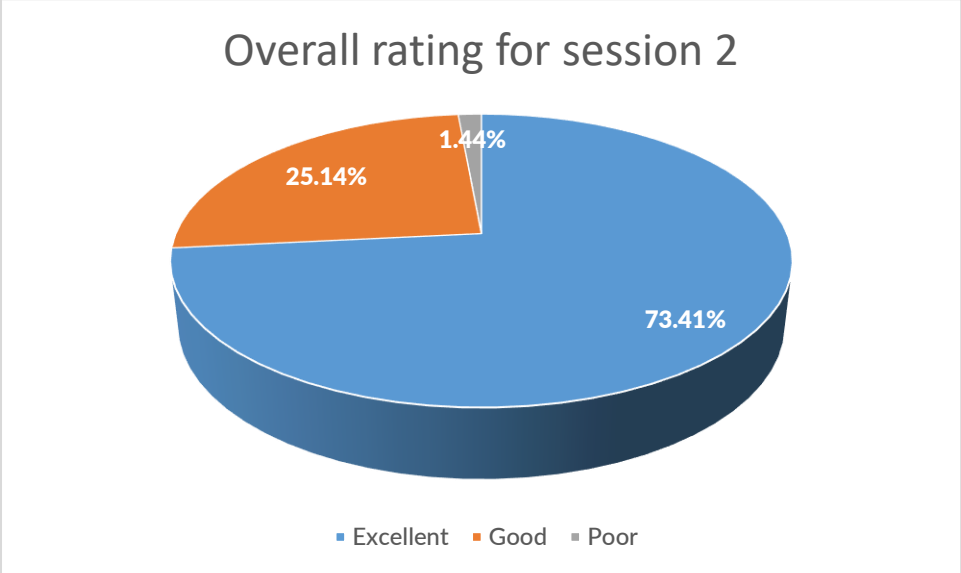
1. 91.07% of delegates rated the *Update: Tyrosine Kinase Inhibitors as a treatment of PH – Lessons Learned and Future Challenges* as 'Excellent' by Ralph Schermuly, University of Giessen.

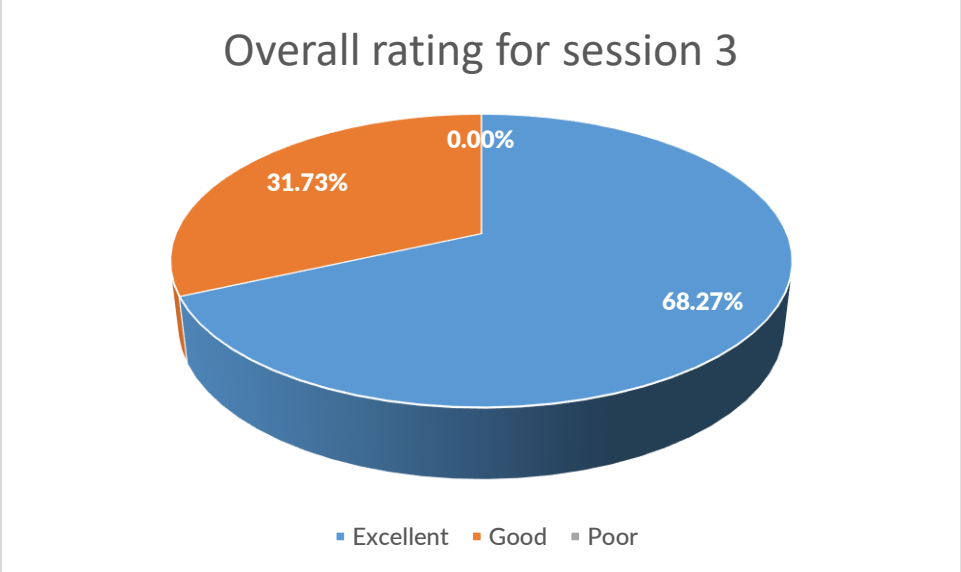
2. 87.50% of delegates rated the *Tocilizumab in the Treatment of Pulmonary Arterial Hypertension (TRANSFORM-UK)* as 'Excellent' by Mark Toshner, University of Cambridge.

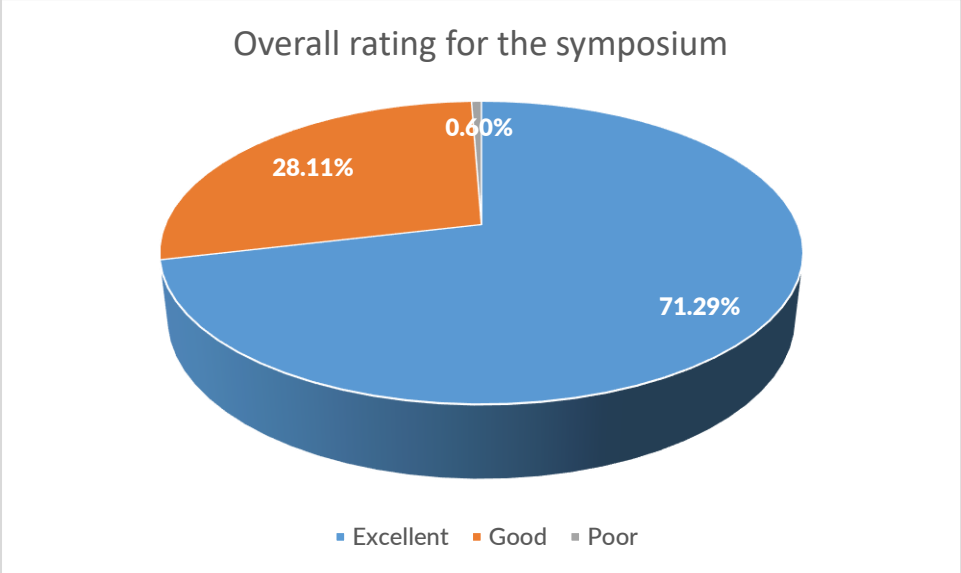
3. 83.33% of delegates rated the *CXA-10 (10-nitro-oleic-acid) for Pulmonary Hypertension* as 'Excellent' by Tanja Rudolph, University of Cologne.

The symposium was divided into 3 sessions, which were rated as follows:

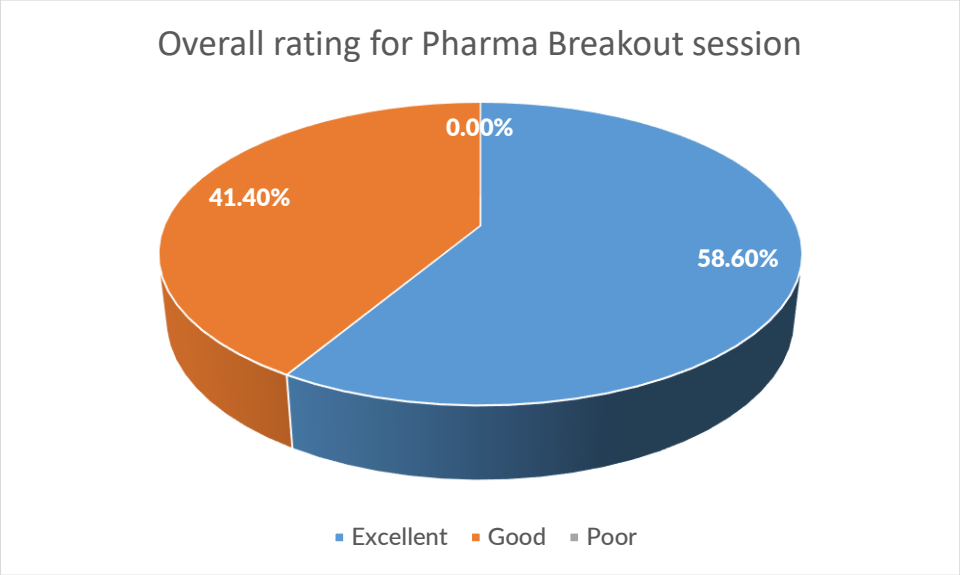



Overall rating for session 2
1.44%
25.14%
73.41%
Excellent
Good
Poor


Overall rating for session 3
0.00%
31.73%
68.27%
Excellent
Good
Poor


Overall rating for the symposium
0.60%
28.11%
71.29%
Excellent
Good
Poor

In addition, the Pharma Task Force held a breakout session, which was rated as follows:



The following table details the ratings for each of the symposium session:

		Overall rating		
		Excellent	**Good**	**Poor**
Day 1 - Session 1 New Drugs for Pulmonary Hypertension Entering Clinical Trials Moderator: John Newman Expert Panel: Marion Delcroix, Nazzareno Galie	A Study of Ubenimex in Patients with Pulmonary Arterial Hypertension: The 'LIBERTY' Trial *Presenter: Norbert Voelkel, VU University Medical Center*	75.00%	23.21%	1.79%
	Tocilizumab in the Treatment of Pulmonary Arterial Hypertension (TRANSFORM-UK) *Presenter: Mark Toshner, University of Cambridge*	87.50%	12.50%	0.00%
	ABI-009 for Severe Pulmonary Arterial Hypertension (WHO FC III and IV) *Presenter: Mark Simon*	58.49%	41.51%	0.00%
	UPDATE: Tyrosine Kinase Inhibitors as a Treatment of Pulmonary Hypertension - Lessons Learned and Future Challenges *Presenter: Ralph Schermuly, University of Giessen*	91.07%	8.93%	0.00%
	Overall rating of session 1	**78.02%**	**21.54%**	**0.45%**
Day 1 - Session 2 Novel Drugs for Pulmonary Vascular Diseases Ready for Clinical Trials	The Serotonin Hypothesis Revisited-Clinical Implications for TPH1 Inhibition *Presenter: Mandy MacLean, University of Glasgow*	70.18%	28.07%	1.75%
	Preclinical Studies of a New Potent Orally Active Antagonist for the MIF-CD74 Axis in Development for Pulmonary Hypertension *Presenter: Marc Humbert, Université Paris-Sud*	70.18%	28.07%	1.75%
	CXA-10 (10-nitro-oleic-acid) for Pulmonary Hypertension *Presenter: Tanja Rudolph, University of Cologne*	83.33%	16.67%	0.00%

		Overall rating		
		Excellent	**Good**	**Poor**
Moderator: **Ardeschir Ghofrani** Expert Panel: **Jean-Luc Vachiery, Laura Price**	Inhaled Sodium Nitrite in Pulmonary Hypertension Associated with Heart Failure with Preserved Ejection Fraction *Presenter: Marc Simon, University of Pittsburgh*	64.81%	31.48%	3.70%
	Demonstration of the PVRI 'Digital Clinic' *Presenters: Martin Johnson, Colin Church, University of Glasgow*	78.57%	21.43%	0.00%
	Overall rating of session 2	**73.47%**	**25.14%**	**1.44%**
Day 2 - Session 3 Moderator: Stuart Rich Expert Panel: Gérald Simonneau, Joanna Pepke-Zaba	Precision Medicine Approach to Rare Disease Therapies *Presenter: Krishna Prasad, Member, Cardiovascular Working Party, The European Medicines Agency*	66.67%	33.33%	0.00%
	UPDATE: Strategies to Alter miRNA Expression that can be Employed in Clinical Trials for Pulmonary Vascular Disease and Right Ventricular Failure *Presenter: Stefanie Dimmeler, University of Frankfurt*	73.08%	26.92%	0.00%
	Peripheral Balloon Pulmonary Angioplasty in the Treatment of CTEPH: Review of the Clinical Experience *Presenter: Christoph Wiedenroth, Kerckhoff-Klinik, Bad Nauheim*	66.67%	33.33%	0.00%
	UPDATE: Implantable Drug Delivery Pumps for Chronic Intravenous Therapies for Pulmonary Vascular Disease and Right Ventricular Failure *Presenter: Manuel Richter, University of Giessen*	66.67%	33.33%	0.00%
	Overall rating of session 3	**68.27%**	**31.73%**	**0.0%**
Pharma Task Force Breakout Session Presenter: Peter Fernandes	Initiative 1 - Harmonise Innovative Approaches Through Clinical Trial Designs, Endpoints, Biomarkers *Peter Fernandes, Sylvia Nikkho, Larry Zisman*	53.57%	46.43%	0.00%
	Initiative 2 - Matching Industry Projects/Interests with Individual Investigator Interests and Core Strengths *Andrew Nelson, Jonathan Langley*	63.64%	36.36%	0.00%
		58.60%	**41.40%**	**0.0%**
	Overall rating for entire symposium	71.29%	28.11%	0.60%

Delegates' comments

- A fantastic event, very interesting, relevant and inspiring!
- Excellent
- Brilliant meeting!
- An excellent symposium!
- Fantastic meeting and venue. Love the intimacy of size with ability to interact with all attendees and meeting staff.
- Excellent meeting. Very honoured to be invited. Really good, frank discussions of clinical trial design, new therapeutics that benefited from industry, research and clinical input.
- Enjoyed the symposium immensely. Liked the format; exceedingly effective and useful for debates. The presentations and participants were very active and this is a strong positive aspect.
- Excellent conference. Learned a lot about state of advances in the field
- In addition to regulators, another key determinant in drug development is payers – consider topic related to implications of trial design on access.
- [The symposium is] in the spirit of breaking down barriers between pharma and academia. Consider adding pharma 'voice' to the expert panel for any development topics. I think it would be informative for all.
- Consider adding formal breaks to enhance networking opportunities.
- Would have like more variety of input from audience those not involved seemed disengaged – seems like a small number of attendees came to the microphone. Would have liked more regulatory discussions as to the future.

Suggestions for future topics and symposium location

- A few more clinically-oriented strategy debates
- Regulatory issues reported to HfpEF/PH would be helpful
- Clinical endpoints for paediatric trials
- FDA input on clinical endpoints – phase II vs III
- PH due to HF1
- More mechanical device.
- Would have like to have a debate, point/counterpoint on 'hot' topics. For examples: CMRI underdevelopment of a drug; single PVRI vs continuous pressure monitoring to evaluate total pressure 'load' as more relevant; ideas for responder analysis 'responses'.
- Would recommend changing to a 2 full day format to allow for morning and afternoon breaks to increase networking opportunities throughout the meeting.