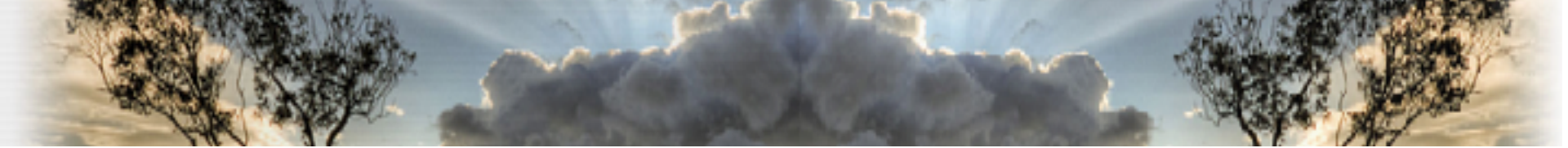
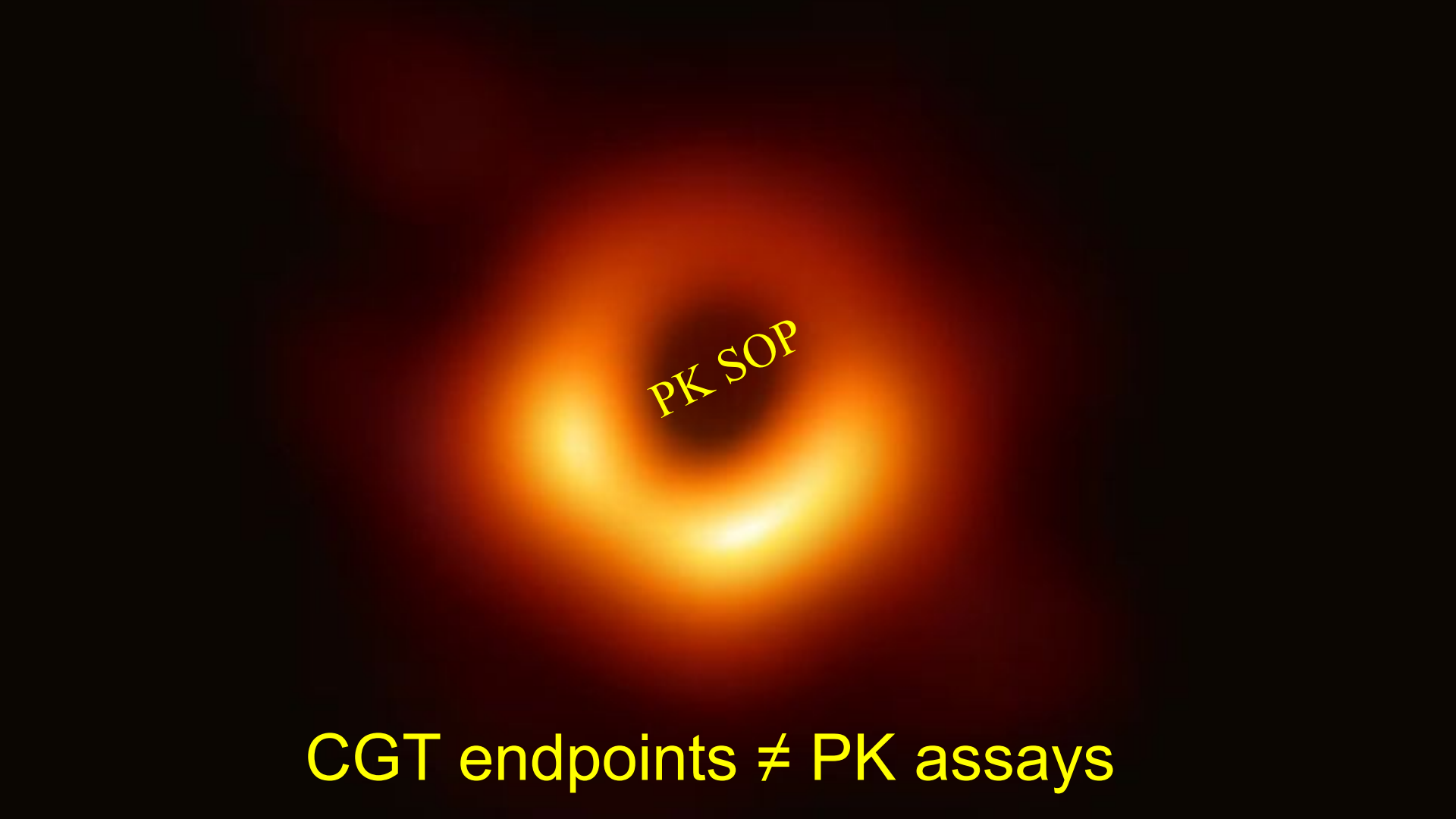


# CGT discussion points from EBF

12th EBF Open Symposium
Imagine! A new bioanalytical Earthrise

http://www.e-b-f..eu




CGT endpoints ≠ PK assays

# Survey results

- Companies currently working on C&GT:
  - Pharma (7) 50%
  - CRO (7) 50%

- Companies not currently in C&GT
  - 28 (of 67)

- Companies who said they will work on it in the future:
  - 3 and growing

# Therapeutic area support

- Immuno oncology (13)
- Genetic disease (8)
- Other. (Please specify):
  - Parkinson’s disease
  - Auto immunity, respiratory
  - Various

| Which phase of drug development do you support? | |
|---|---|
| Discovery | 29% |
| Non-clinical studies | 36% |
| Clinical studies | 50% |
| Post marketing | 7% |
| All phases | 50% |

| Where do you look for regulatory guidance? | |
|---|---|
| Bioanalytical method validation guidance (FDA, EMA, …) | 93% |
| Clinical Pathology (CLIA, CLSI, CAP…) | 43% |
| White papers/publications (EBF, AAPS, …) | 100% |
| Internal | 64% |
| Other, please specify: | Guidelines on gene and cell therapy (clinical, non-clinical) |

**Do you think current Bioanalytical regulations are sufficient for you to support Cell and Gene therapy studies?**

86% - NO

EBF

# Where do you think the EBF community can contribute in this space?

| | |
|---|---|
| Gap analysis of BMV | 71% |
| Regulatory interaction and training | 57% |
| Shape future regulatory landscape | 86% |
| Hands on laboratory protocols | 29% |
| Best practice documents | 79% |
| Other: | • Work with therapy developers to shape what's required<br>• Harmonisation / standardisation of BA and safety assessment. |

# Workshop and conference discussions

What is the role of the BioA lab and BioA scientist in supporting these drug developments?

- Vision of bioanalytical lab to bring together all necessary expertise together from a scientific and regulatory perspective
- Requirement to get BioA lab involved at an early stage
- Some companies have dedicated C&GT groups; not all activities performed in house but specialised out-sourced activities monitored by same group

# Bring CGT in the BioA world, including regulations?

- No current regulatory guidance. Good opportunity to bring in scientific rationale on how assays should be validated – general opinion that ‘fit-for-purpose’ was a good approach
- Harmonisation is needed and EBF can facilitate that
- Work can fall between current BMV and patient management regulations (AAV nAb as inclusion criterion, CAR-T persistence and immunophenotyping)
- Good science should be the primary driver and the necessary regulations applied
- It is better to have no regulation than a bad regulation

# Adapt existing regulations or introduce new regulation?

- Every company appears to have their own BioA strategy – rather than try to standardise through regulation. EBF as a platform to share experience and regulatory feedback
- Harmonisation is needed but a publication strategy is better than regulatory documents
- Clarify in relevant parts of current BioA guidance to include CGT endpoints
- ICH currently working on CGT guidance – Let's ensure BioA gets a seat at the table
- Learn from BioA strategies that have been used for licensed gene therapies
- Mixed opinion on whether separate regs are required for CGT or BioA technologies

# BioA community concerns?

- Amount of investment for new technologies for relatively small sample numbers – CGT often aimed at rare populations with low numbers of patients in trials. Often granted orphan drug status so approval is accelerated
- Gaps in BioA strategy need to involve all stakeholders at start of development program to identify and prioritise BioA requirements
- For cell therapies, the physical distance of patients from the lab in order to provide meaningful results
- Uncertainty if cell therapy exposure and persistence assessments by flow are really BioA endpoints
- Additional handling capabilities required in labs for handling GMOs

# Future direction

- Training Day on 12 May 2020 in Malaga
- Platform for sharing assay and regulatory experiences
- Further discussions at the EBF strategy meeting in March

# Acknowledgment (alphabetical)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- Manuela Braun – Bayer
- Robert Nelson – Covance

Non-EBF member

- Paula Miranda – Uniqure

- Survey participants
- Workshop participants



Thank You !

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