BEBPA 2024 U.S. Bioassay Conference

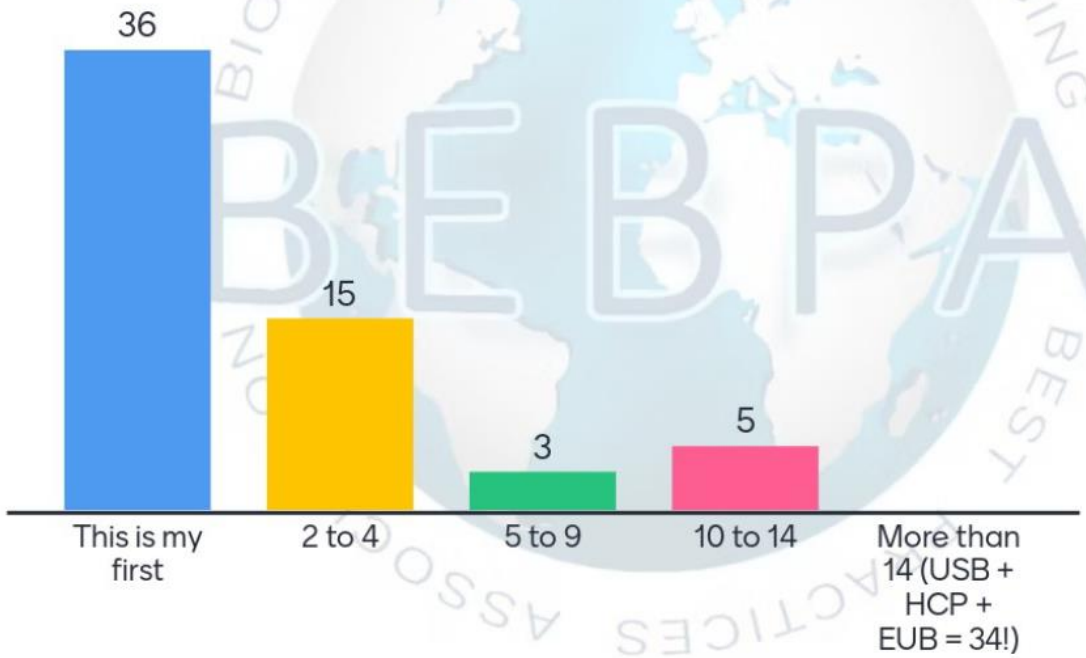
11-13 March 2024
Long Beach, CA, USA
Hybrid Event

Welcome Back & Introduction

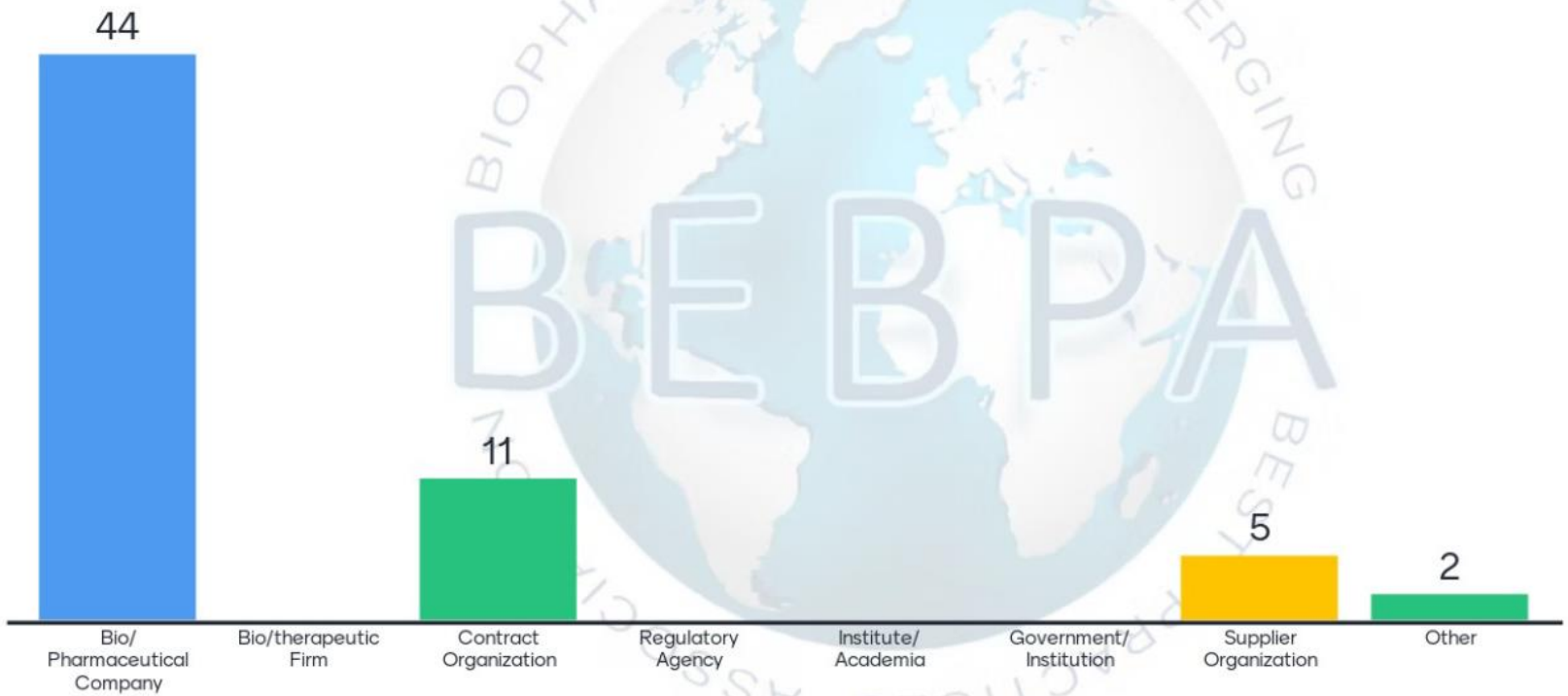
Laureen Little
Principal Consultant
Quality Services
BEBPA President

Audience Surveys

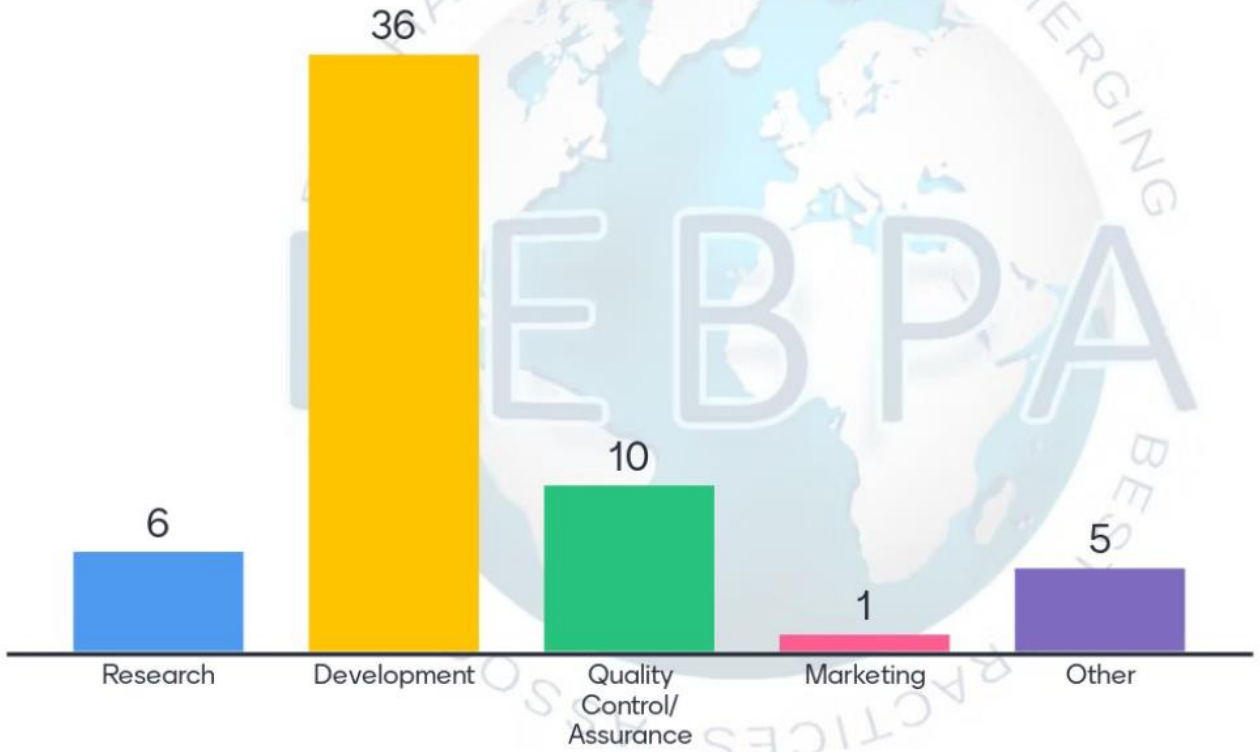
i.1 How many BEBPA Conferences have you attended?



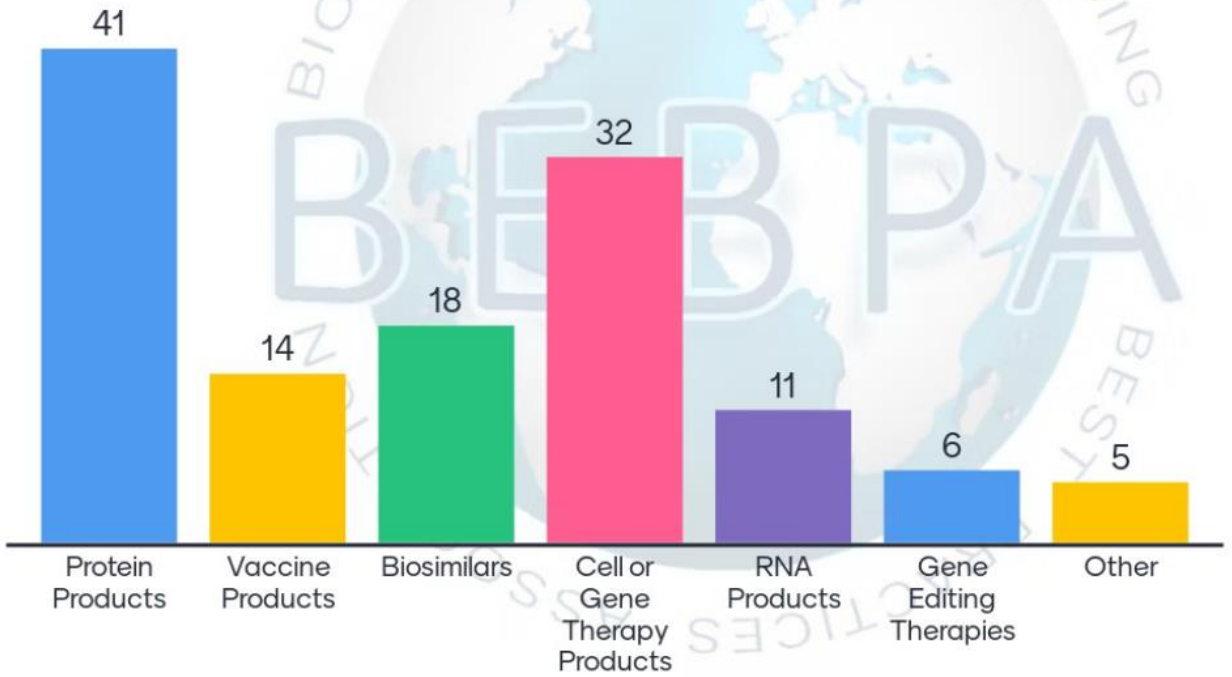
i.2 What type of organization do you work for?



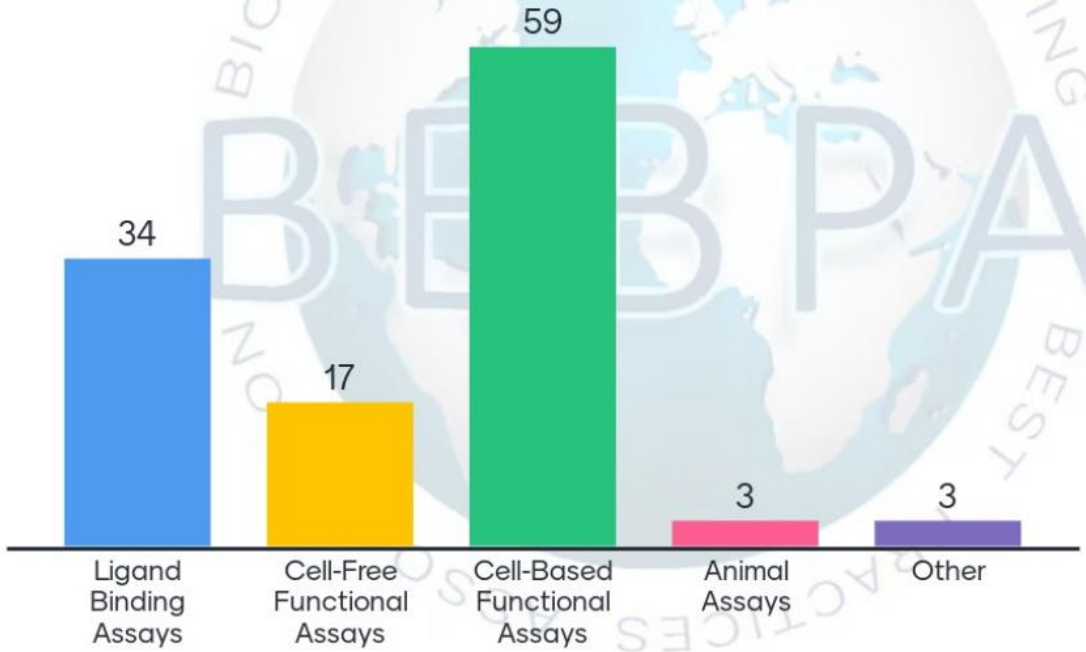
i.3 What part of the organization do your work for?



i.4 What type of products do you work with? (Check all that apply)



i.5 What type of assays do you develop? (Check all that apply)

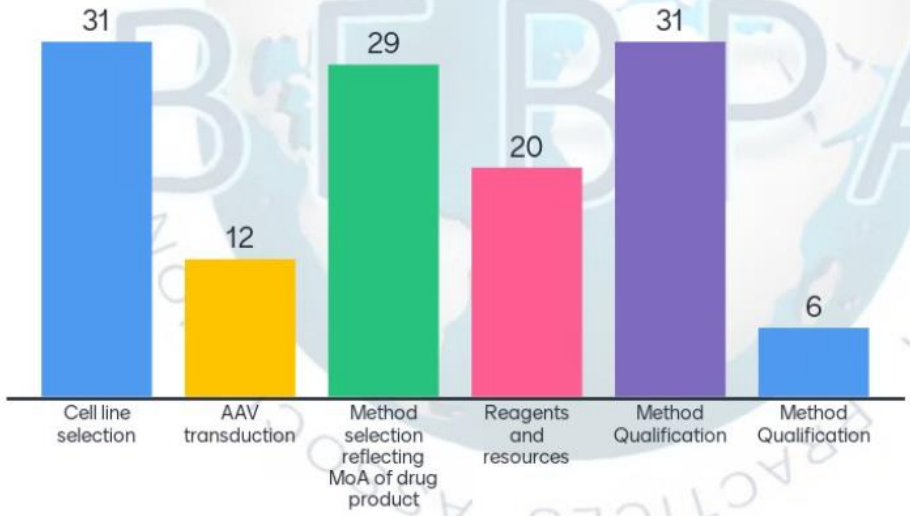


Session 1: Lifecycle Assay Development

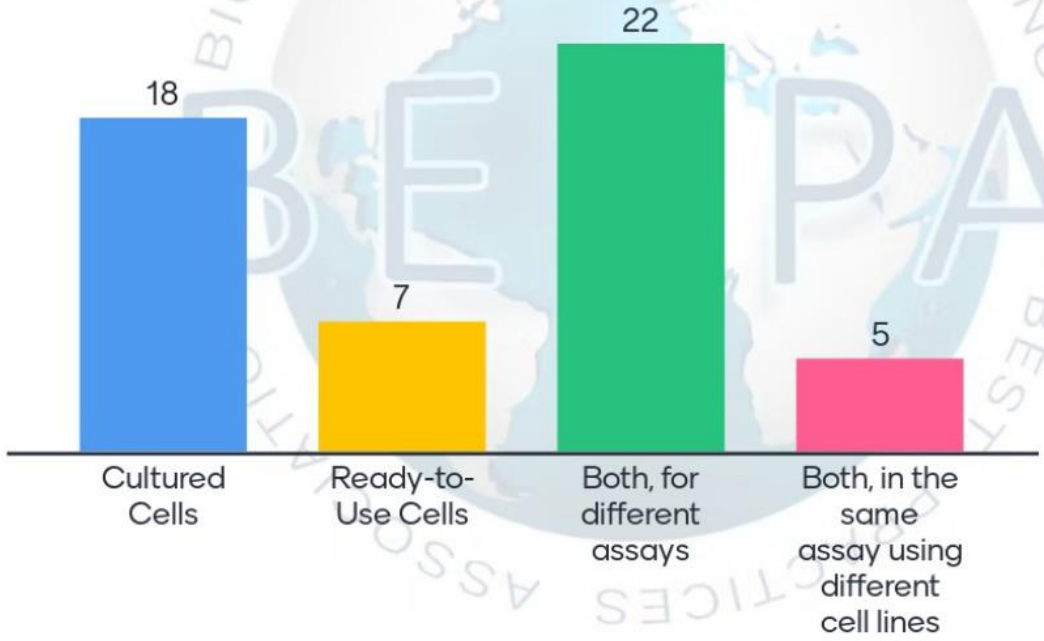
Session Chair: Laureen Little
Principal Consultant
Quality Services
BEBPA President

Audience Surveys

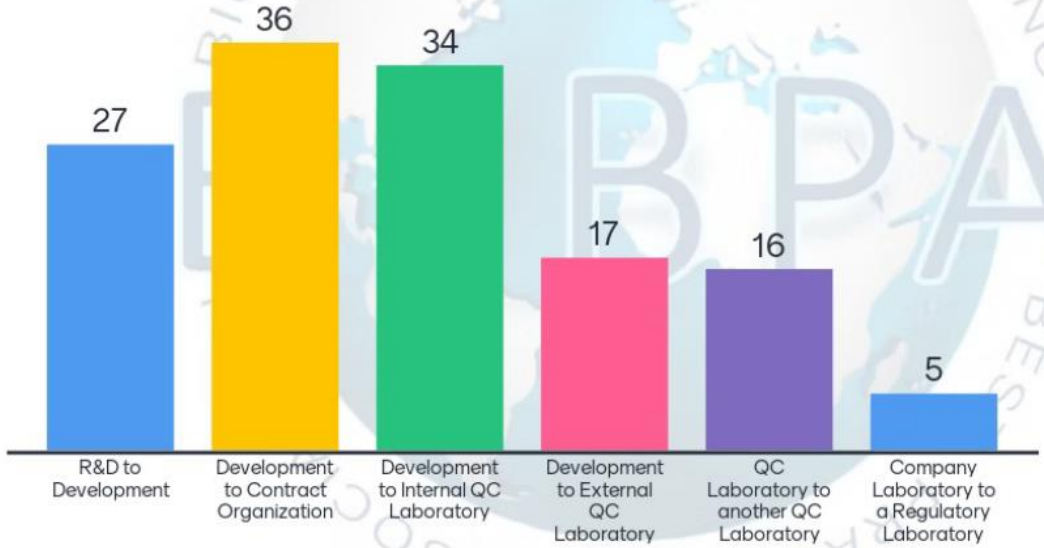
1.1 What are the challenges that you faceed during development, qualification and validiation of cell-based potency assay for gene therapy products?



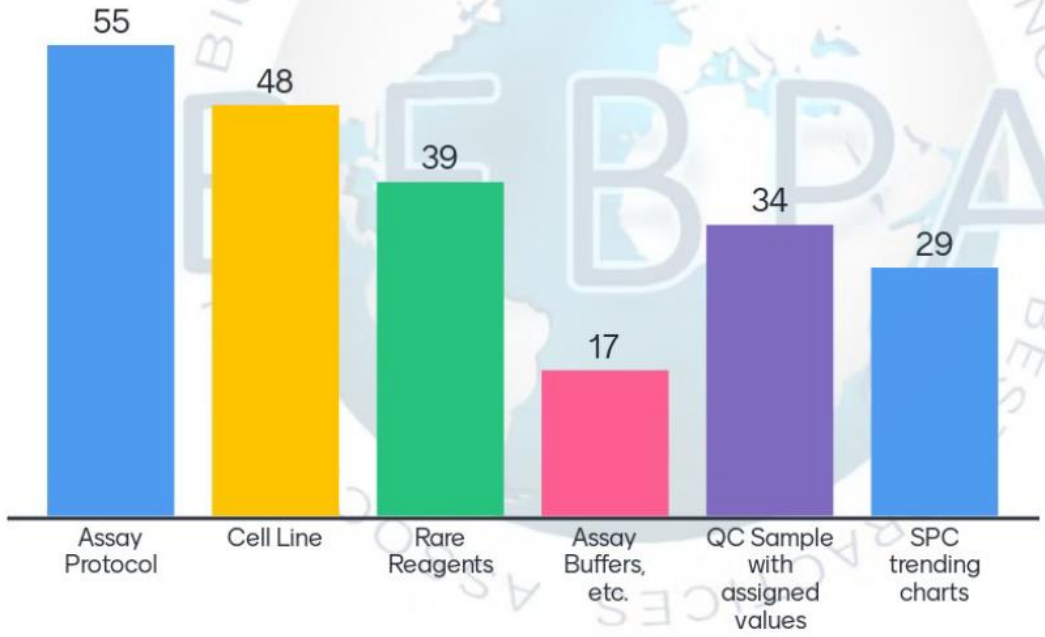
1.2 For your validated potency assays, which of the following do you use?



1.3 What potency assay transfers have you been involved with?



1.4 Which data do you like to send (or receive) when you are transferring assays?

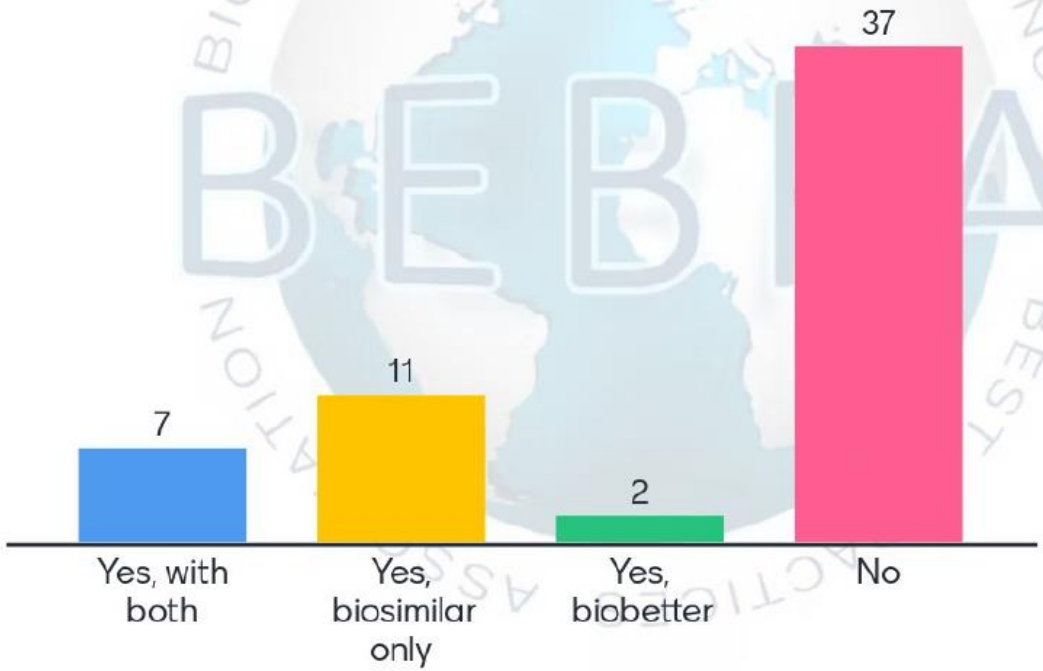


Day 2 Audience Surveys

Session 2A: Biosimilar Potency Assay Development
Session 2B: Managing your Potency Assays in the Real World

Session Chair: Dorota Bulik
Senior Director
Ultragenyx

2.1 Have you every worked with a biosimilar or biobetter (or 2nd generation) products



2.2 What new technology are you interested in learning about?

AI

AAV

Dealing with non-traditional dose/response curves

Automation
Automation
Automation
Automation
Automation
Automation
Automation
Automation
Automation
Automation
Automation

Biophysical characterization

ddPCR
ddPCR

Viral entry

Infectivity
Infectivity

Software

Microfluidics

Biosimilar and biobetter methods

I know it all.....

Functional assays
Functional assays

Lentivirus

Cell & gene therapy potency assays

PBMC ADCC

Single cell analysis
Single cell analysis

Lipid nanoparticle delivery assays

SPR
SPR
SPR
SPR

Promega growth factors assays

New potency assay methods & development

Similarity assessment

Organ on a chip
Organ on a chip
Tissue on a chip
Tissue on a chip

Potency assays for cell gene mRNA therapies

Potency Assay Development

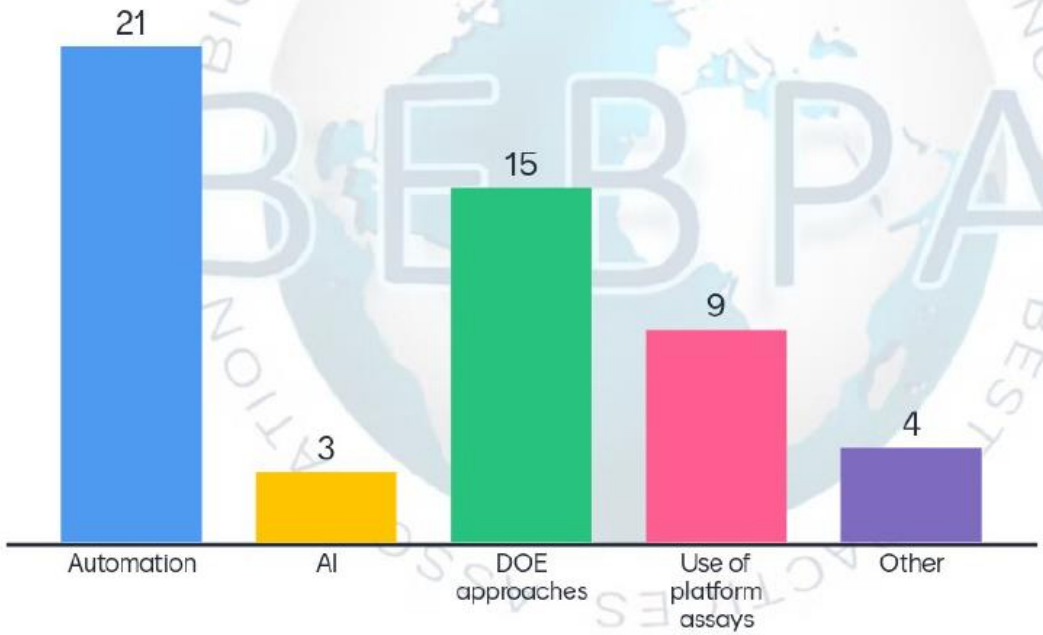
RTU Cell Line generation

Potency assay criteria for working/primary reference standard implementation

11

35

2.3 What tools do you use to improve assay efficiency?

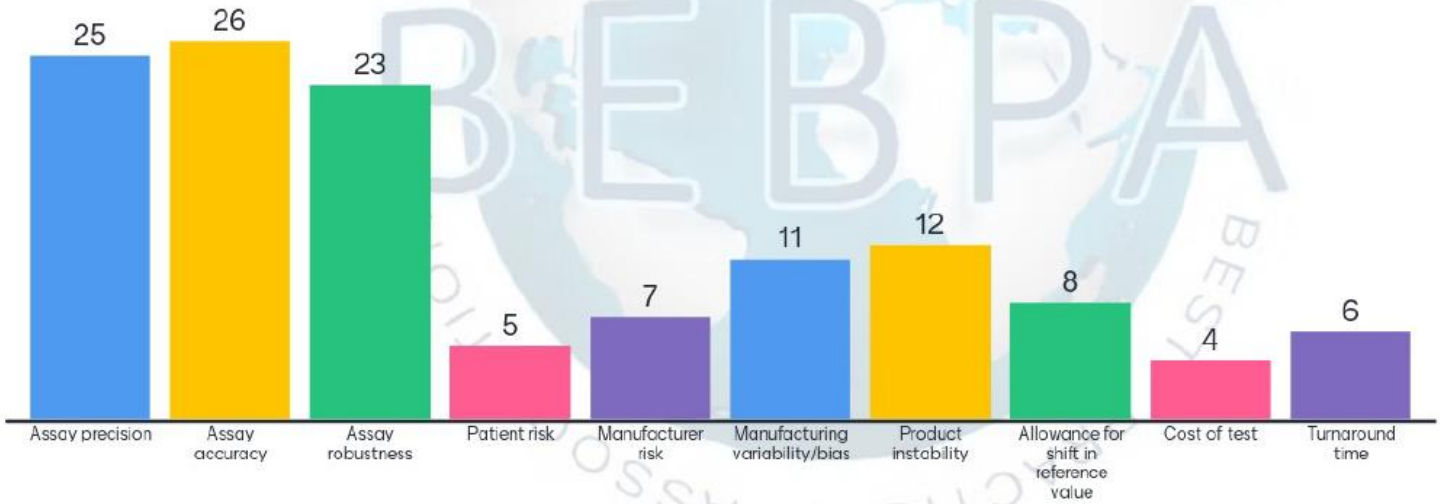


Interest Group 1: Data Analysis

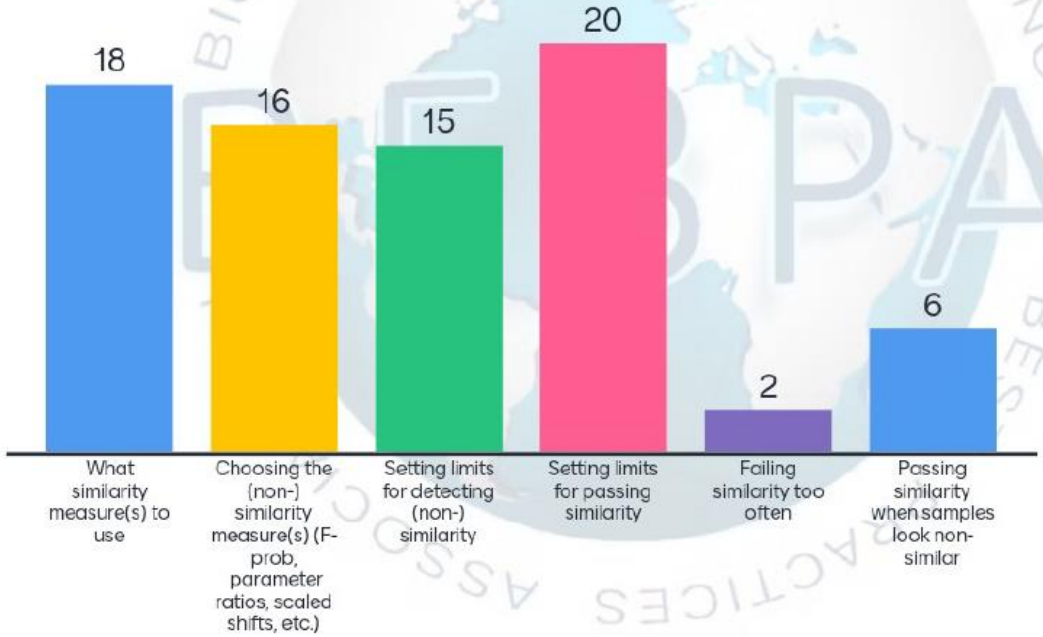
Interest Group 1 Leader:
Nancy Niemuth
Statistical Consultant
Act Two Consulting
BEBPA Bioassay Scientific Committee

Audience Surveys

IG1.1 What elements do you quantify/evaluate in establishing lot release acceptance limits for potency during development?



IG1.2 What are the similarity challenges in your bioassay(s)?



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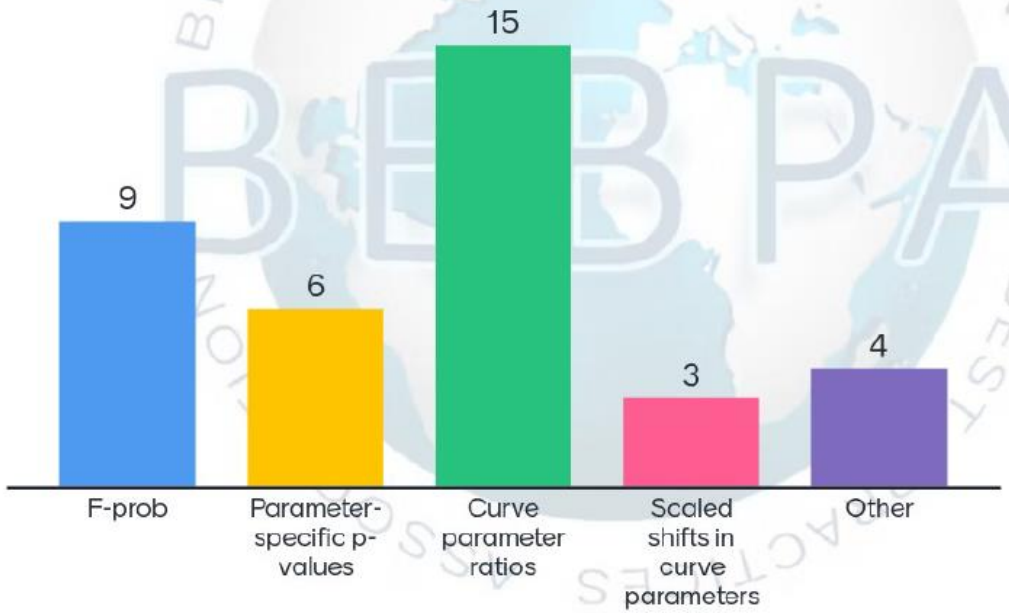
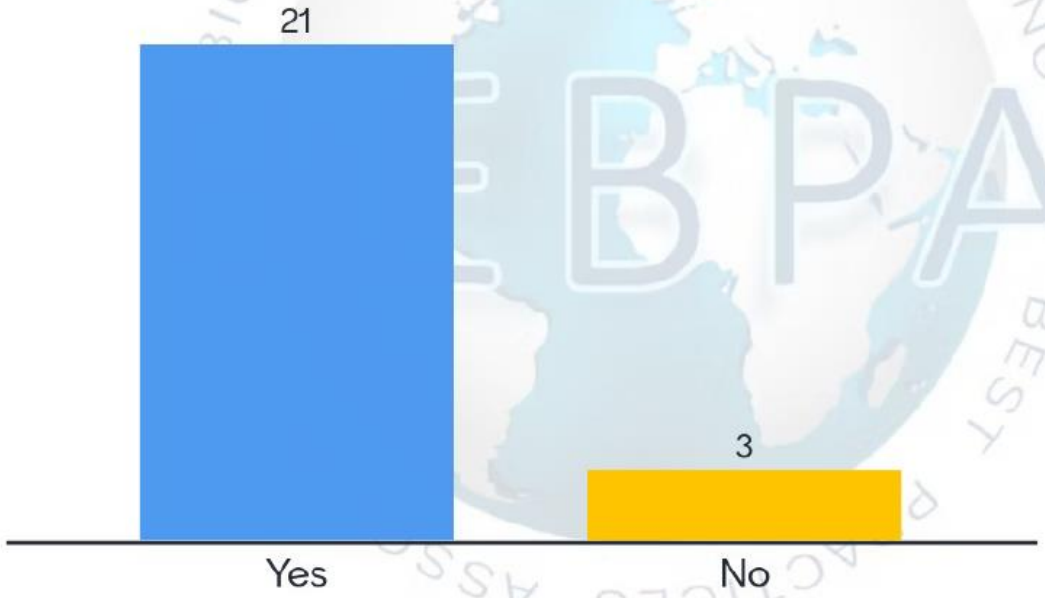
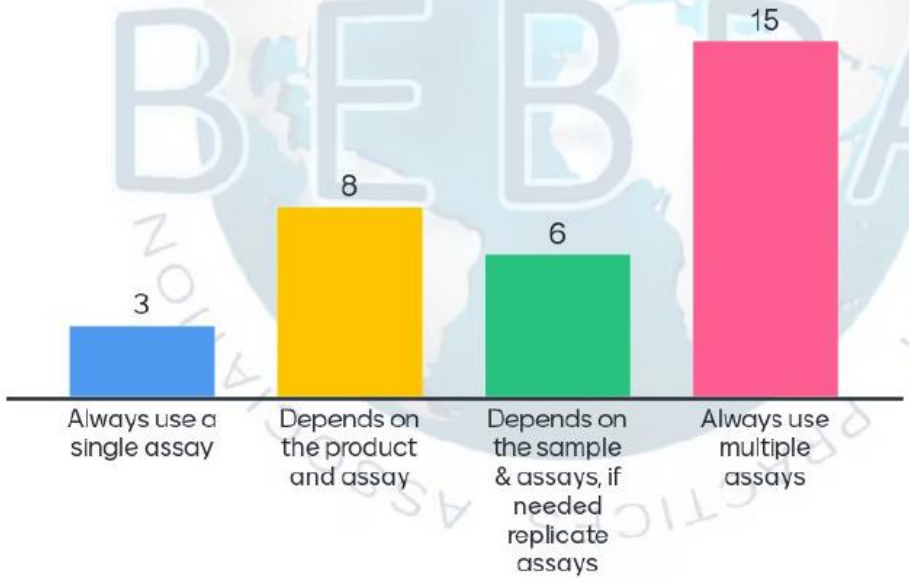
IG1.3 What (non-) similarity measures do you use?



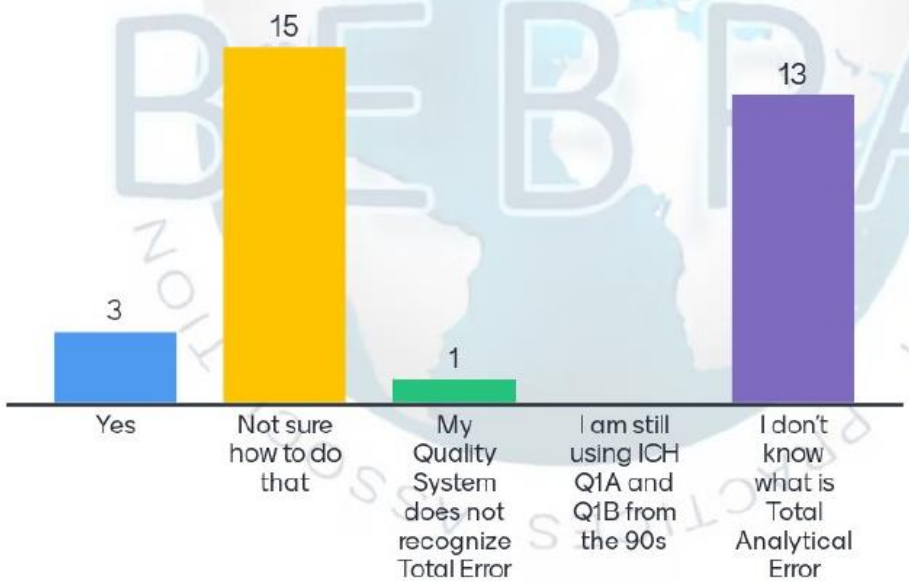
IG1.4 Do you have more than one test sample in each assay?



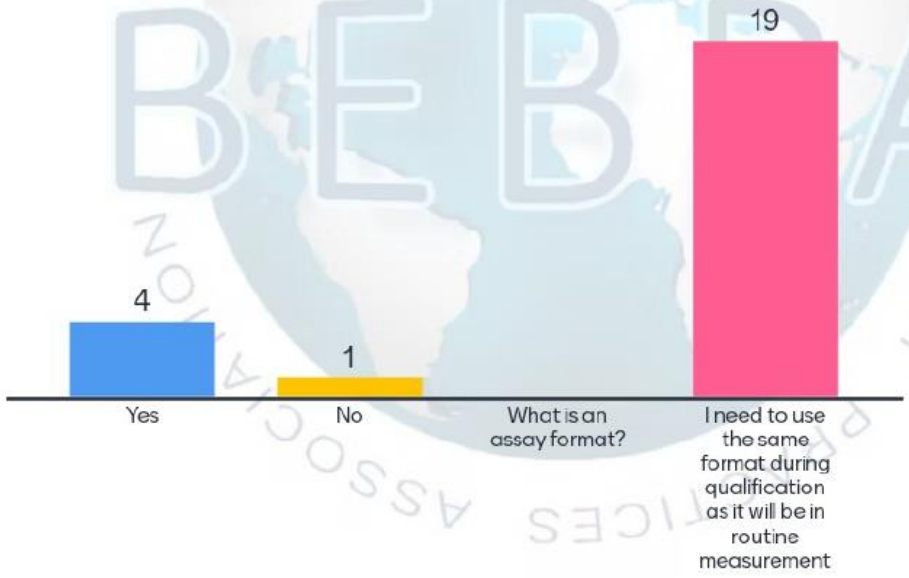
IG1.5 Do you report potency (compare a lot to a product or release specification) based on a single assay or a summary from multiple assays?



IG1.6 I have written a Total Error Analytical Target Profile (ATP) based on the product specification (QTPP):



IG1.7 My procedures allow me to define an assay format to match my ATP, after I obtained my assay qualification/validation results

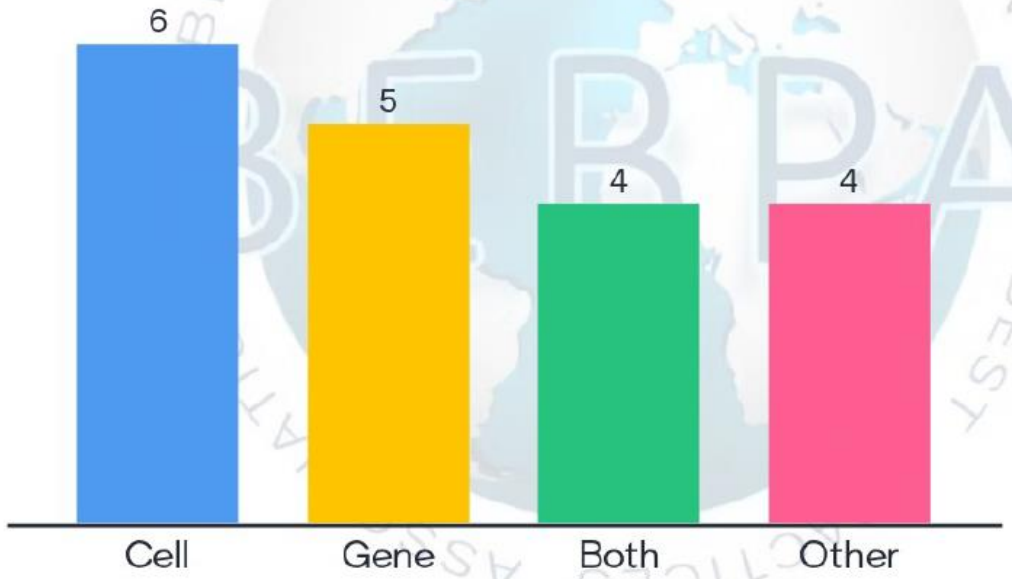


Interest Group 2: Assuring Potency for Cell and Gene Therapy Products

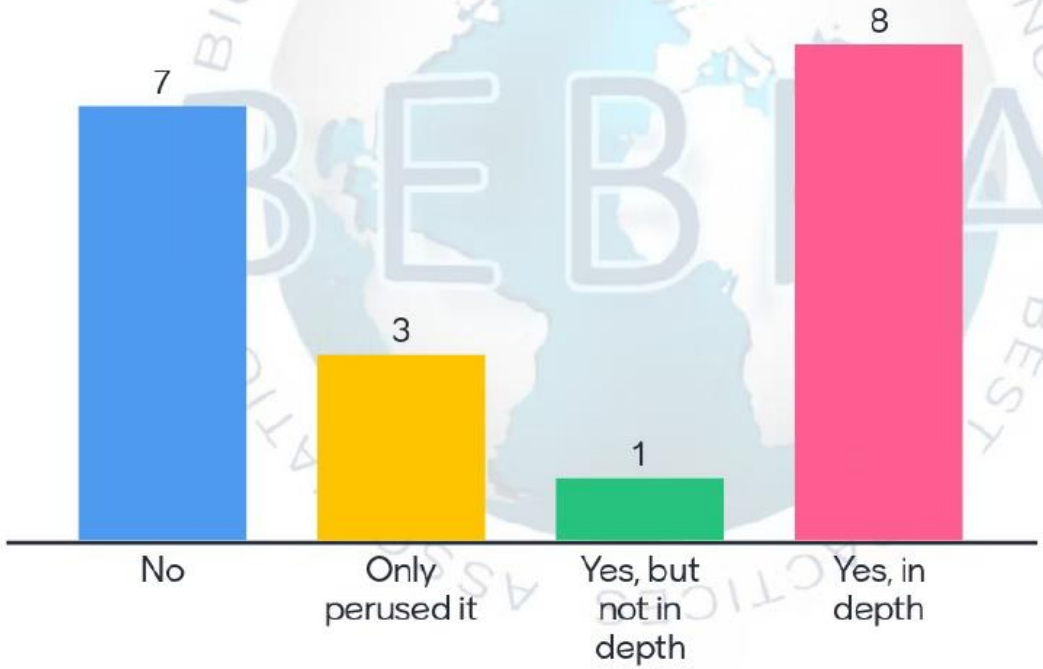
Interest Group 2 Leaders:
Mike Sadick, Senior Director, Imugene
Laureen Little, President, BEBPA

Audience Surveys

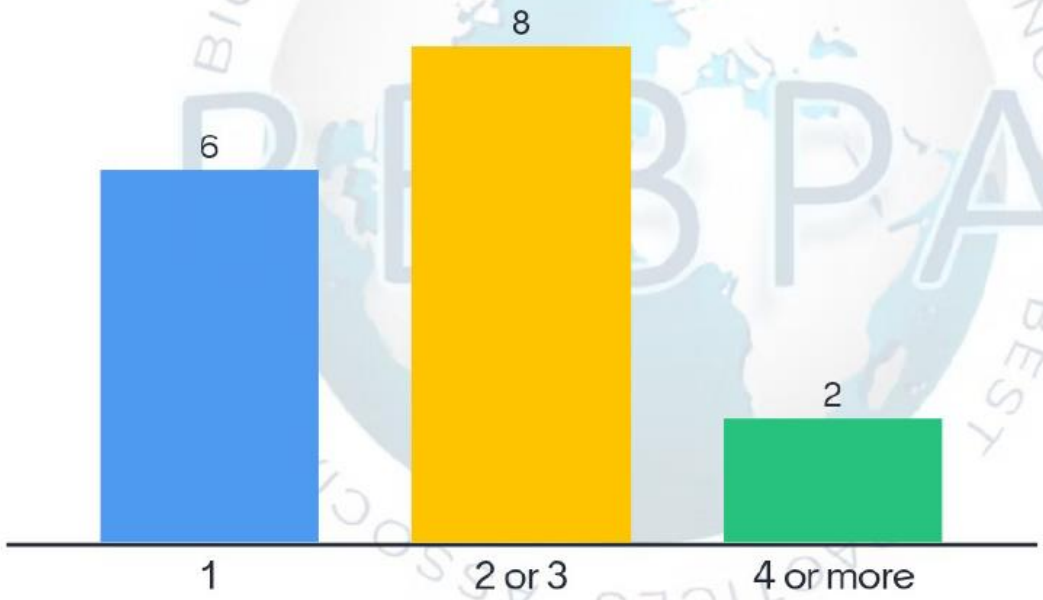
IG2.1 Are you working on a gene or cell therapy?



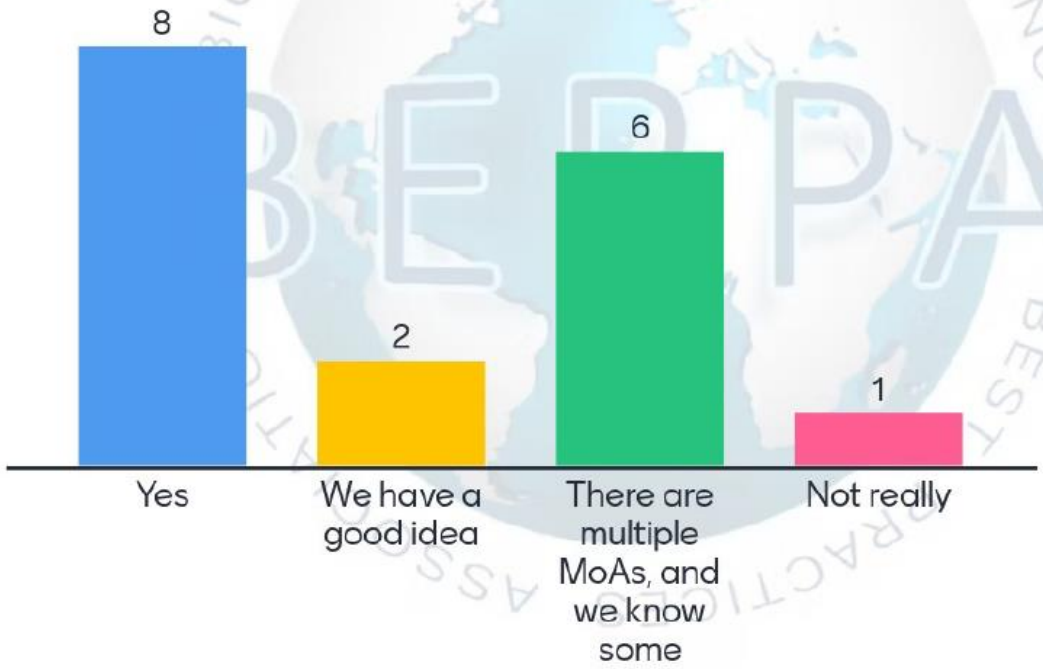
IG2.2 Have you read the newest CGT potency guidance?



IG2.3 How many potency assays are you developing per product?



IG2.4 Do you know the Mechanism of Action of your therapeutic?

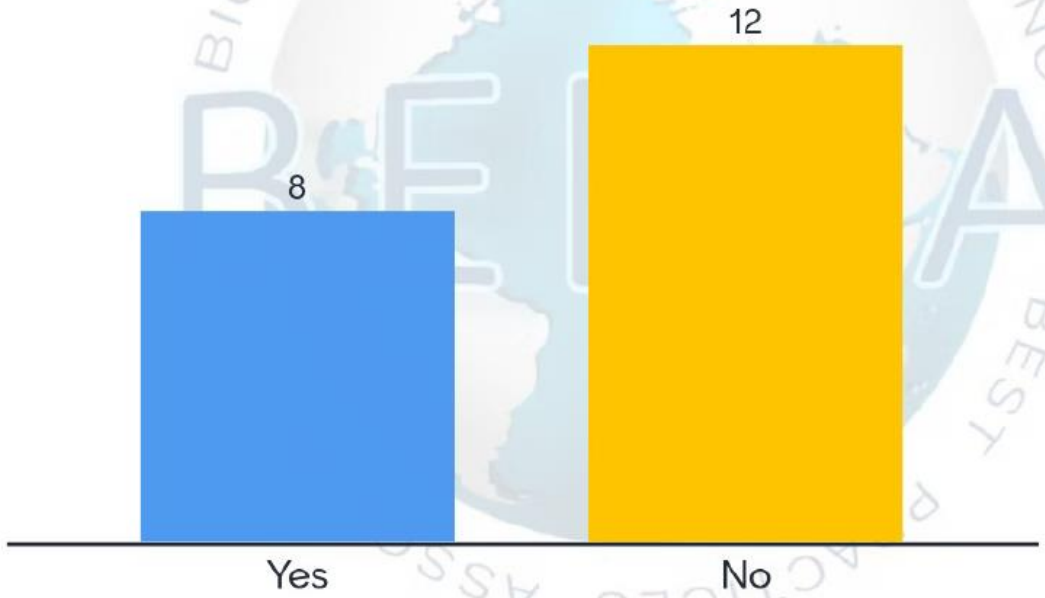


Interest Group 3: Handling Dose-Response Curves

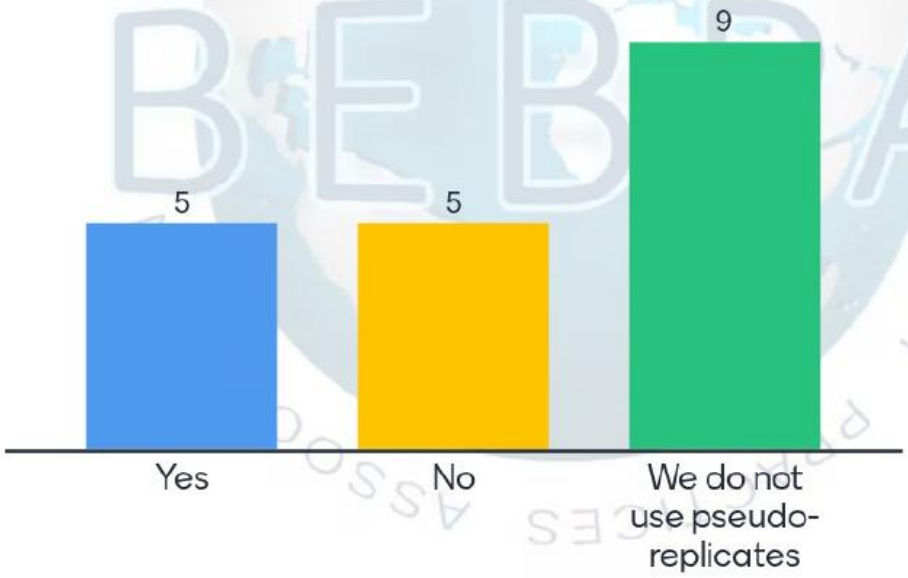
Interest Group 3 Leader:
Anton Stetsenko
Director, Analytical Development
Orca Bio
BEBPA Bioassay Scientific Committee

Audience Survey

IG3.1 Do you use pseudo-replication in your assays?



IG3.2 If you use psuedo-replication, do you average the pseudo-replicates before fitting a statistical model?

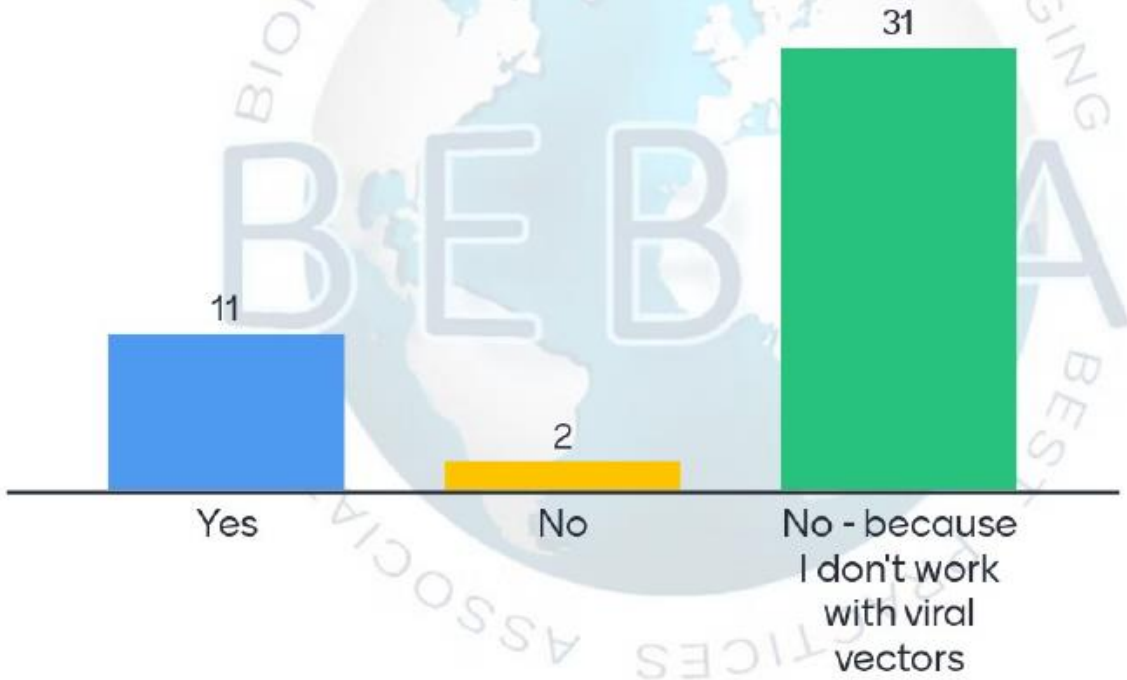


Day 3 Audience Surveys

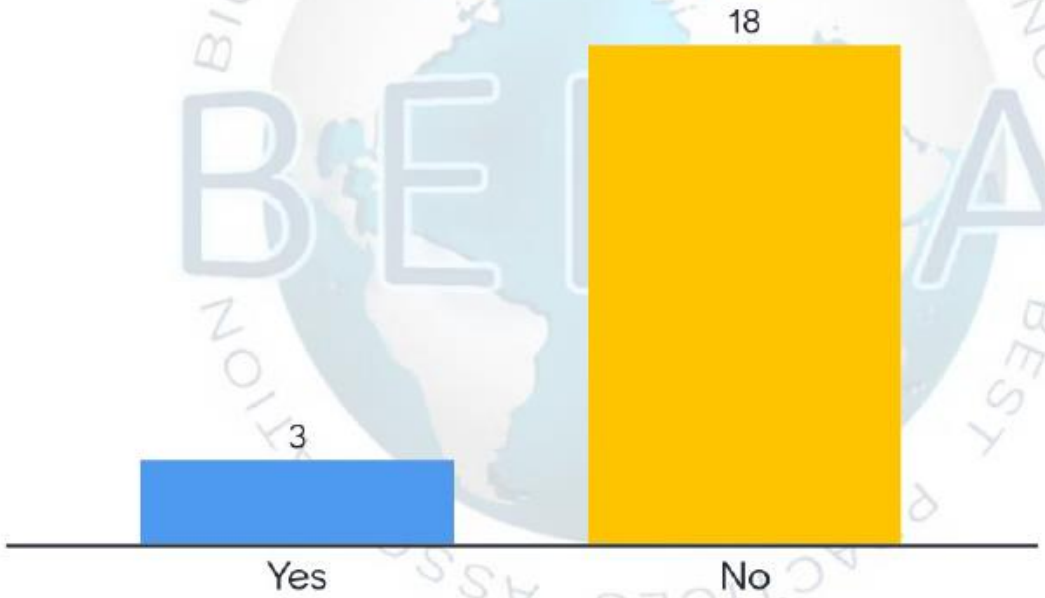
Session 3A: Reference Material
Session 3B: Rapid-Fire Talks

Session Chair: Kristin Clement
Director of Analytical Development
Roivant Sciences
BEBPA Bioassay Scientific Committee

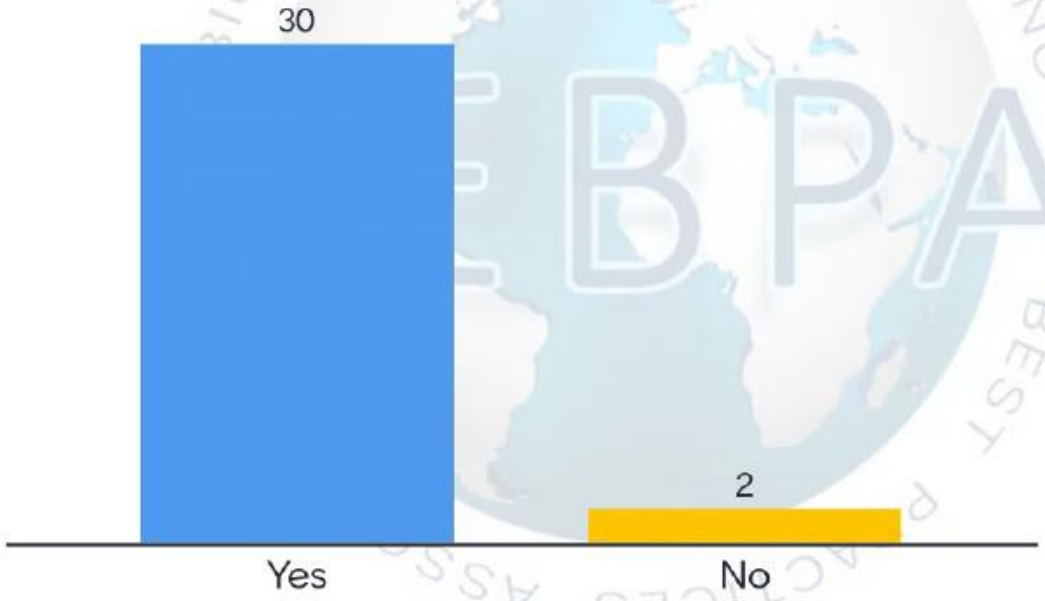
3.1 Would you use VCN reference materials for vector copy number measurement if available?



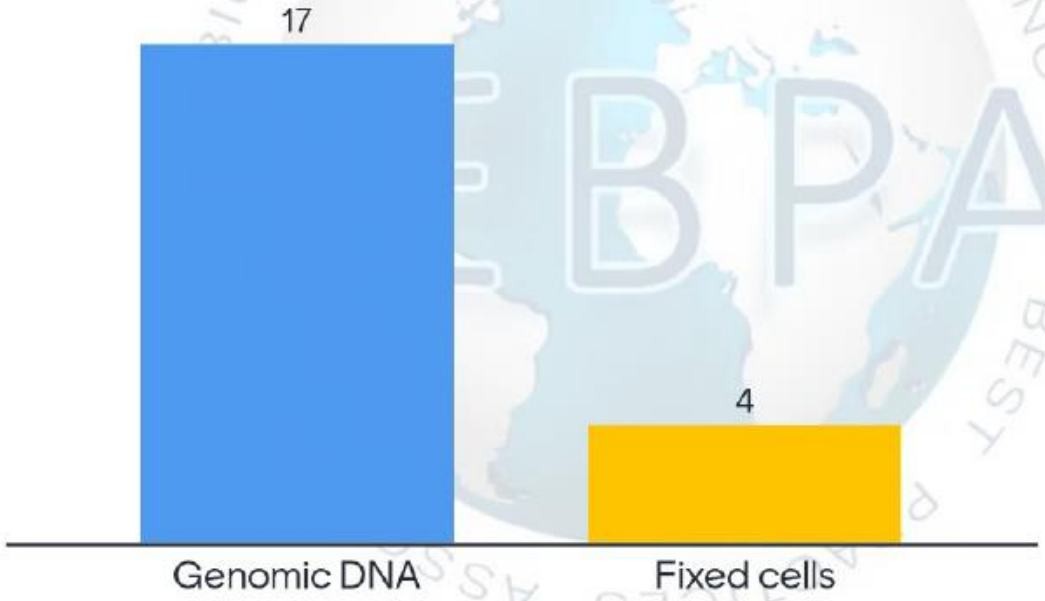
3.2 Is greater than 5 copies VCN reference materials needed?



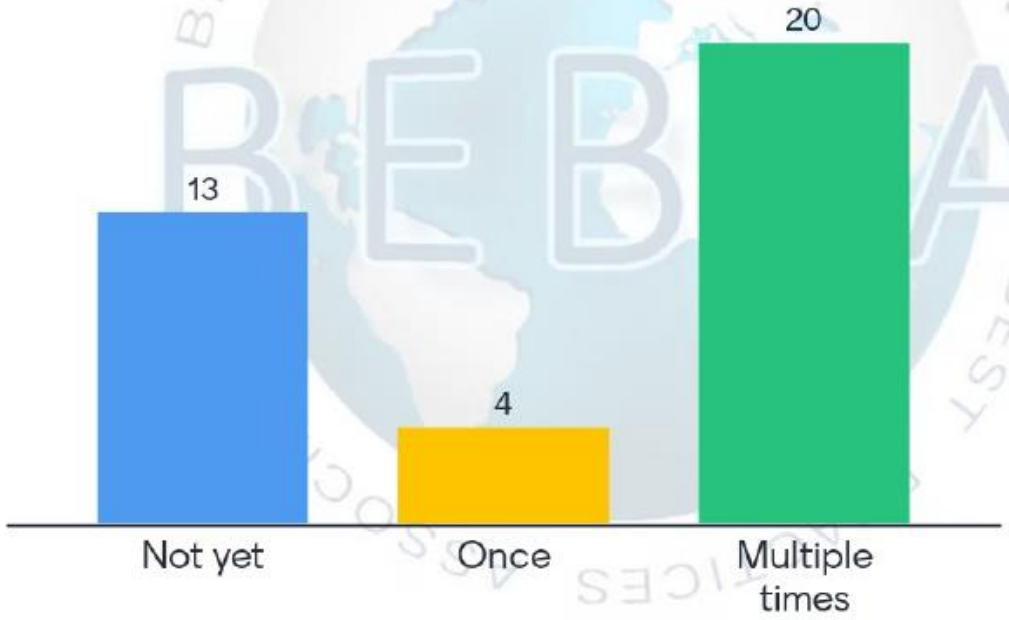
3.3 Is functional killing CAR-T reference material needed?



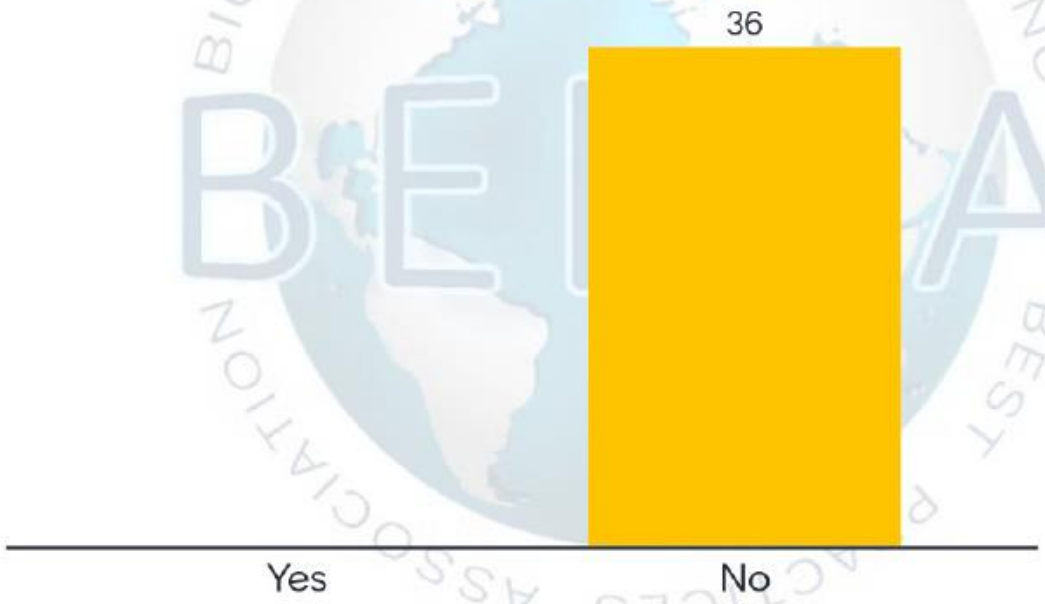
3.4 Which format of VCN reference materials do you prefer?



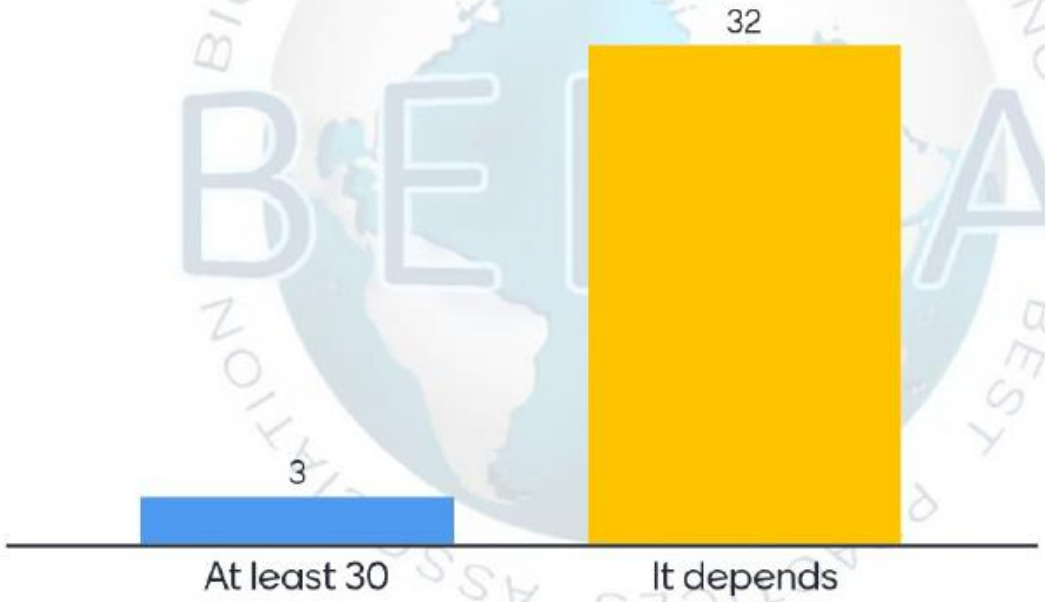
3.5 Have you switched your Reference Standard?



3.6 Do you switch your Reference standard at the same time as your Assay control?



3.7 How many runs do you need to switch Reference standard?

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Thank you!!