CPC COMBINATION PRODUCTS COALITION

CPC Open Door Forum:

Combination Products Guidance Survey and 2008 Policy Agenda

January 22, 2008

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The CPC would like to thank RAPS for their invaluable assistance with this survey, and for the generous use of their survey software.

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Agenda

1. Brief background on the CPC
2. Today's purpose – validation step
3. Survey purpose, scope and methodology
4. Survey results
    a. Demographics
    b. Satisfaction with existing guidance
    c. Need for FDA guidance – what and why
5. Impact on 2008 policy agenda

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CPC: Mission

- Develop and advocate for public policies on behalf of combination product industry
- Forum for building bridges among drug, device, and biological product industries
- Work collaboratively with FDA on combination product issues

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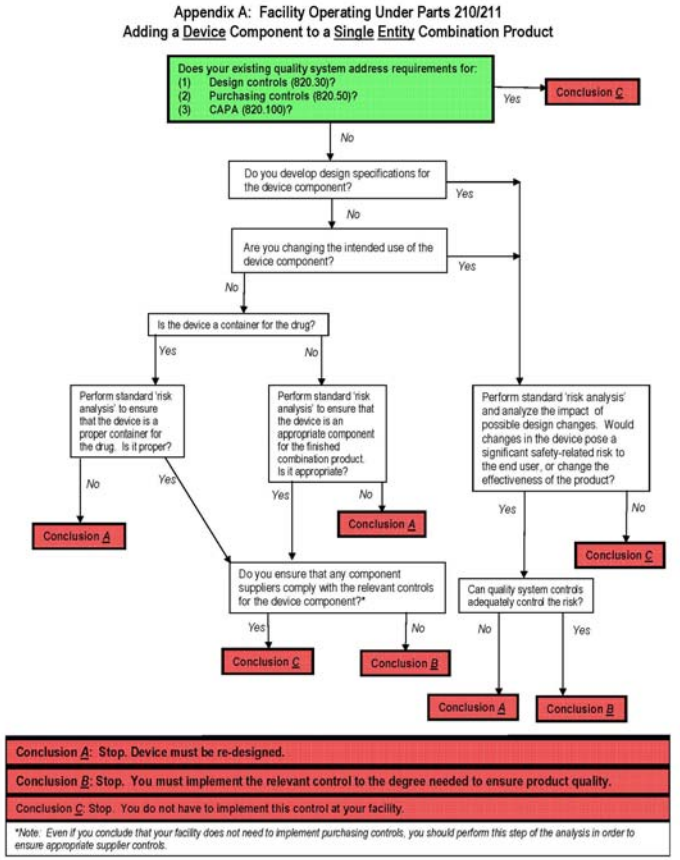
Activities

- Developing and advocating for consensus policy positions
- Examples:
  - Submitted dozens of written comments, policy documents, proposed guidance documents, and other materials to FDA
    - GMP draft guidance and algorithms
    - Adverse Event Concept Paper
    - Cross-labeling algorithm
  - Planning joint conference with RAPS on GMPs to be held when proposed rule is released
  - Partnered with RAPS to host January 2005 Summit attended by approx. 150 people that resulted in a consensus white paper submitted to FDA



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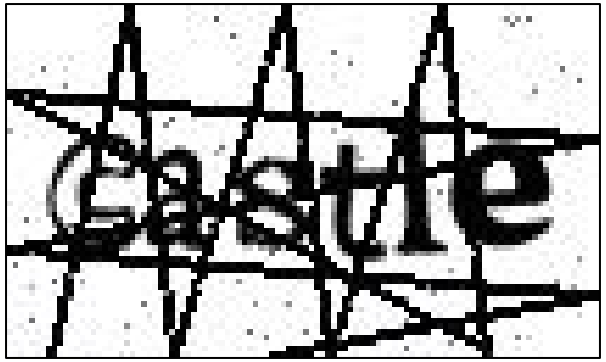
Validation

- Opened this webcast to combination products industry
- Validation of survey data and their impact on 2008 policy agenda



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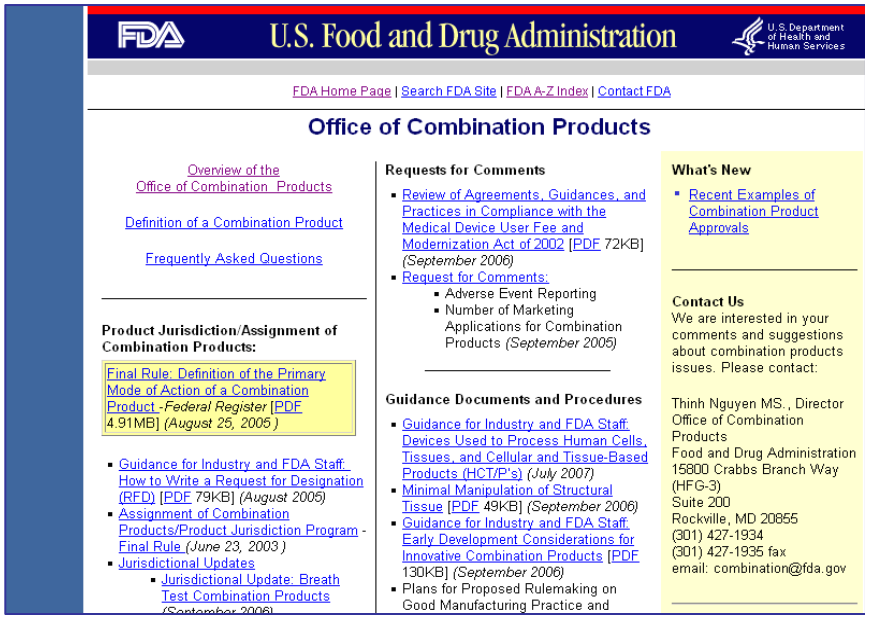
Survey Purpose

- Goals
  - Evaluate current industry concerns and priorities
  - Inform our 2008 policy agenda
  - Communicate these to FDA



- Why now?
  - In 2007, the OCP underwent several leadership and personnel changes; new permanent director effective Jan. 7, 2008
  - Also wanted to take a step back and reflect on our activities

Survey Scope

- Focused questions on:
  - Demographics
  - Satisfaction with existing guidance (FDA and non-FDA)
  - Topics on which more or better FDA guidance is needed
    - What type is needed
    - Why guidance is needed
- Allowed free-form comments

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Survey Methodology

- Disseminated widely to the combination products industry
  - Our individual contacts and through trade groups and publications
  - Many thanks to the organizations that helped get the word out:
    - California Healthcare Institute
    - IMDMC
    - *MD&DI*
    - MDMA
    - *MX*
    - NEMA
    - PharmaMedDevice
    - RAPS

- Respondents completed survey via an Internet link that allowed them to remain anonymous (providing identifying information was optional)
- Asked companies to complete only one survey, but to collaborate with their colleagues

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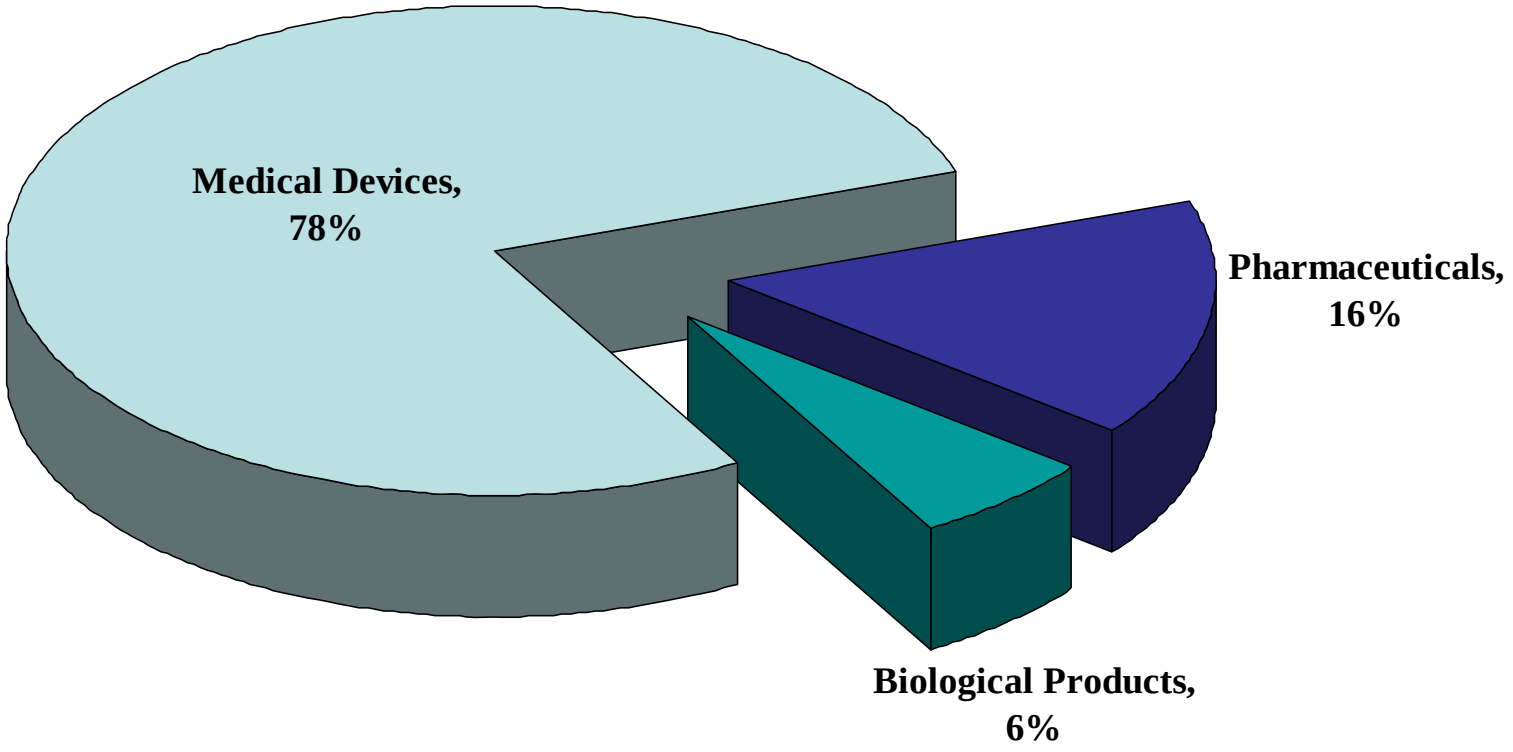
Primary Product Focus



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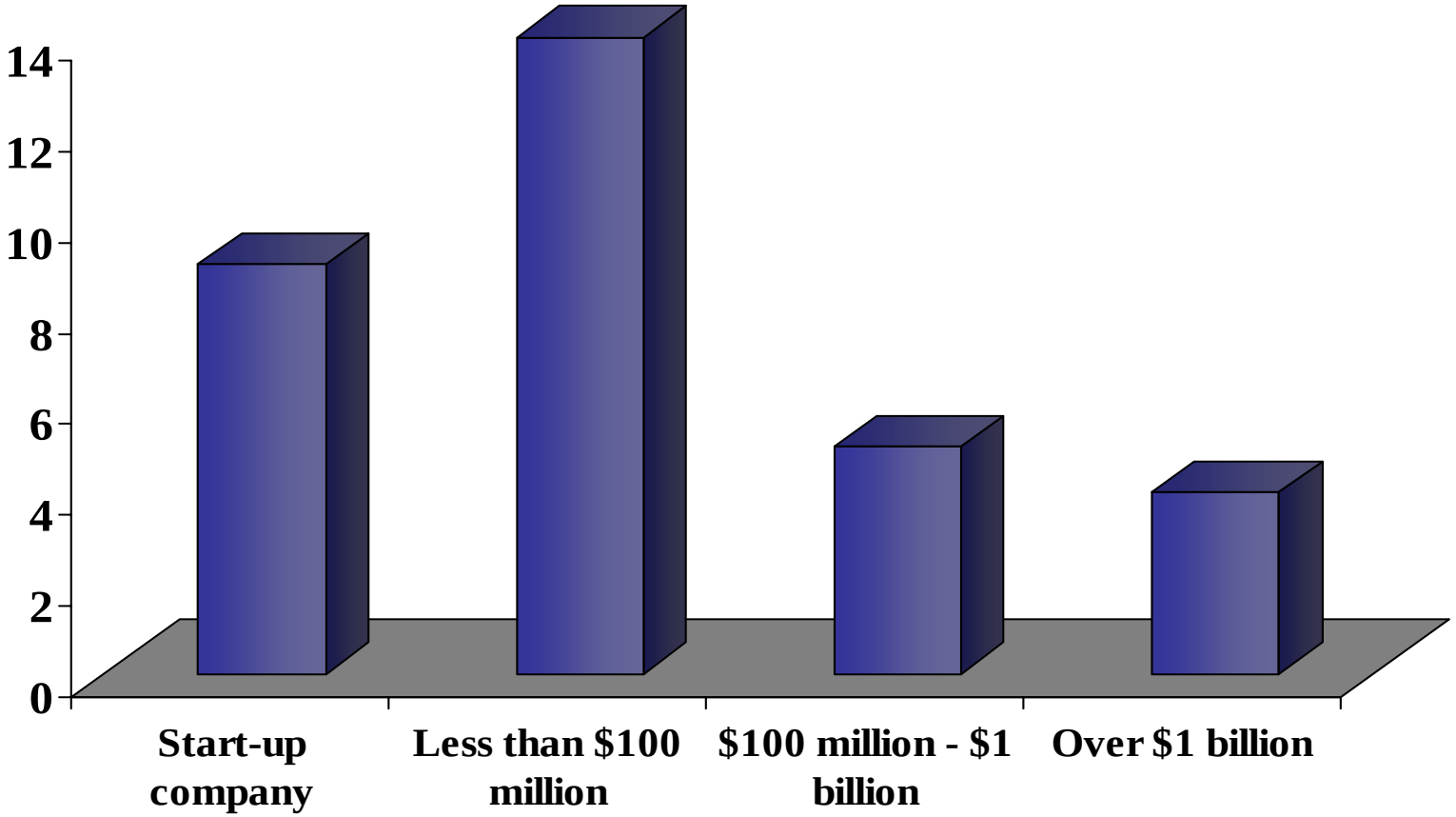
Annual U.S. Sales of Combination Products



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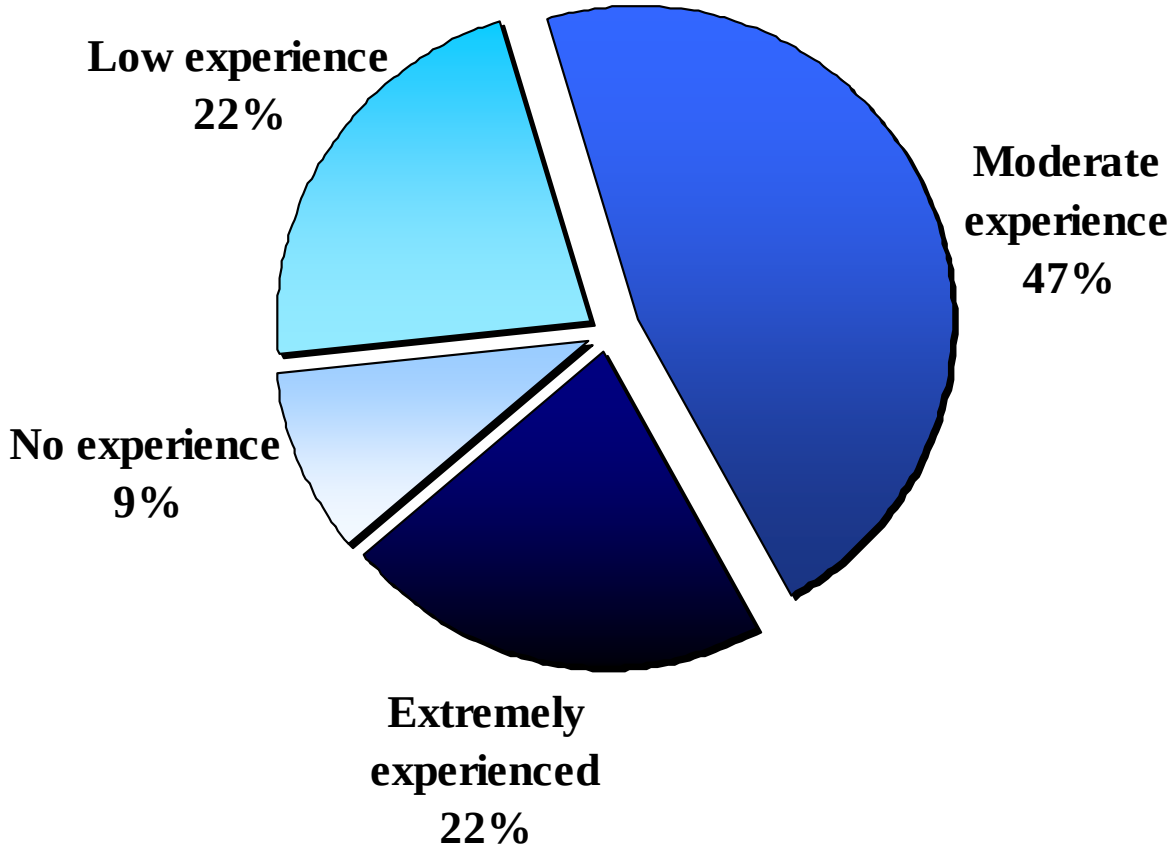
Level of experience with developing and commercializing combination products



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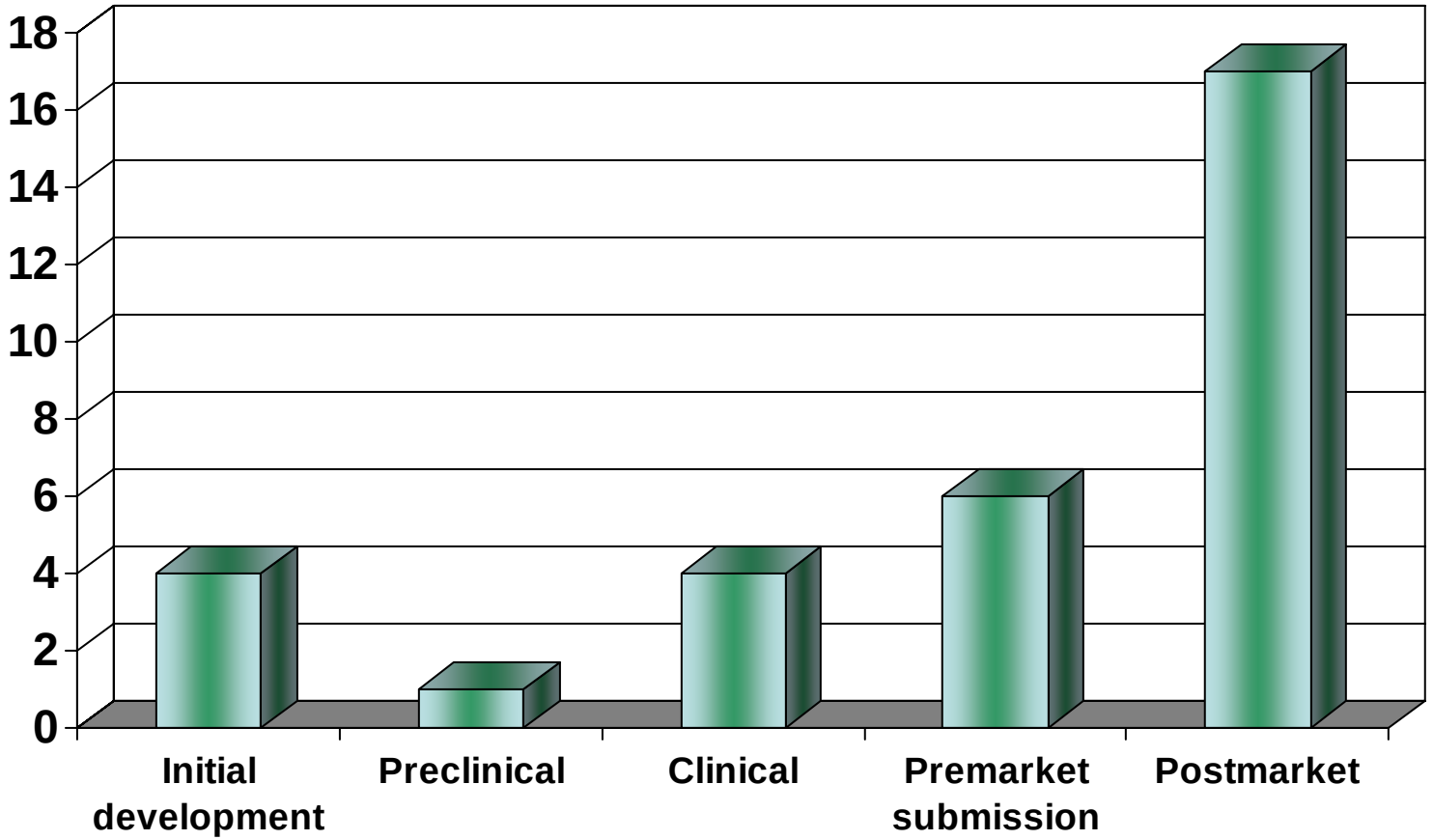
Stage of development of combination product that is furthest along



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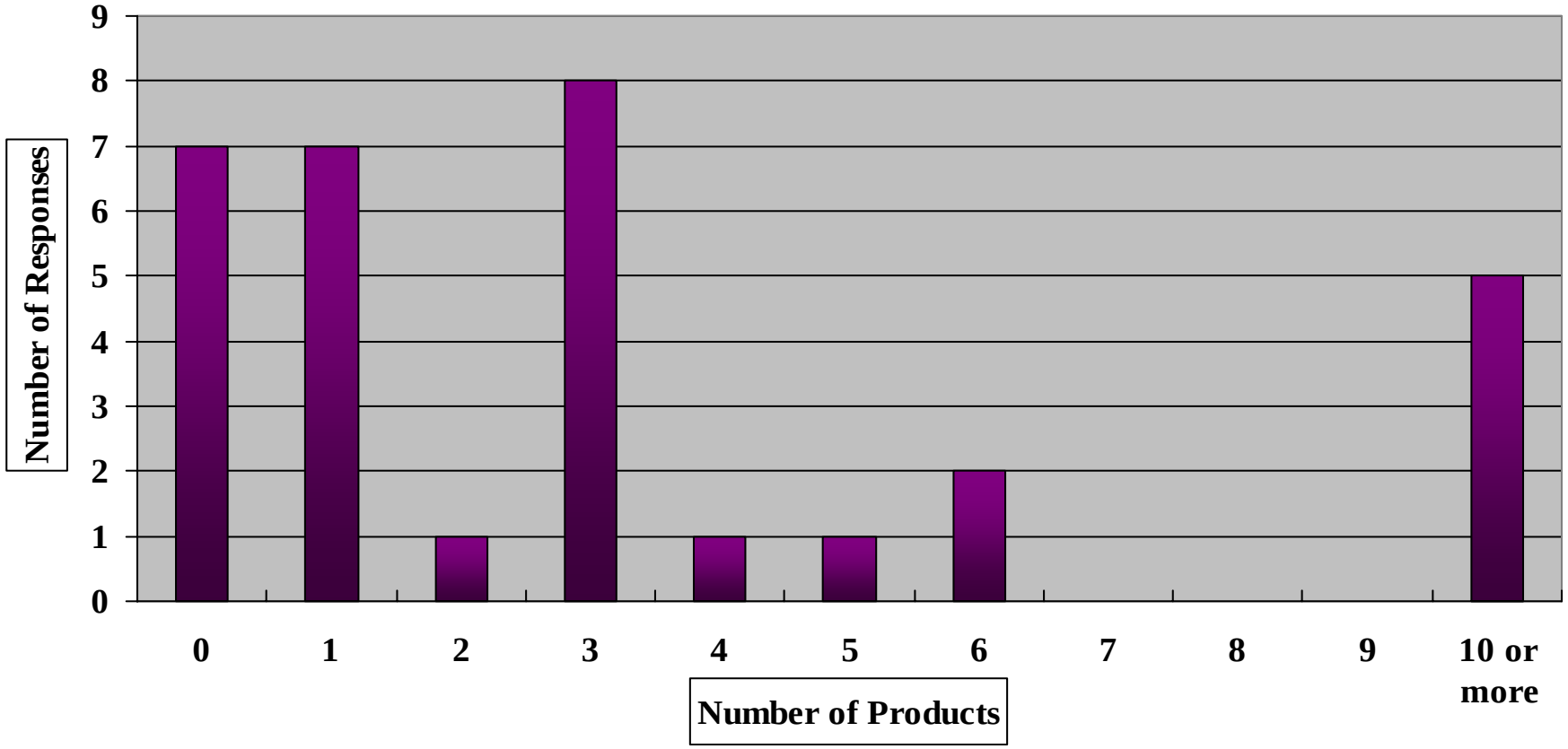
Number of products developed and brought to market



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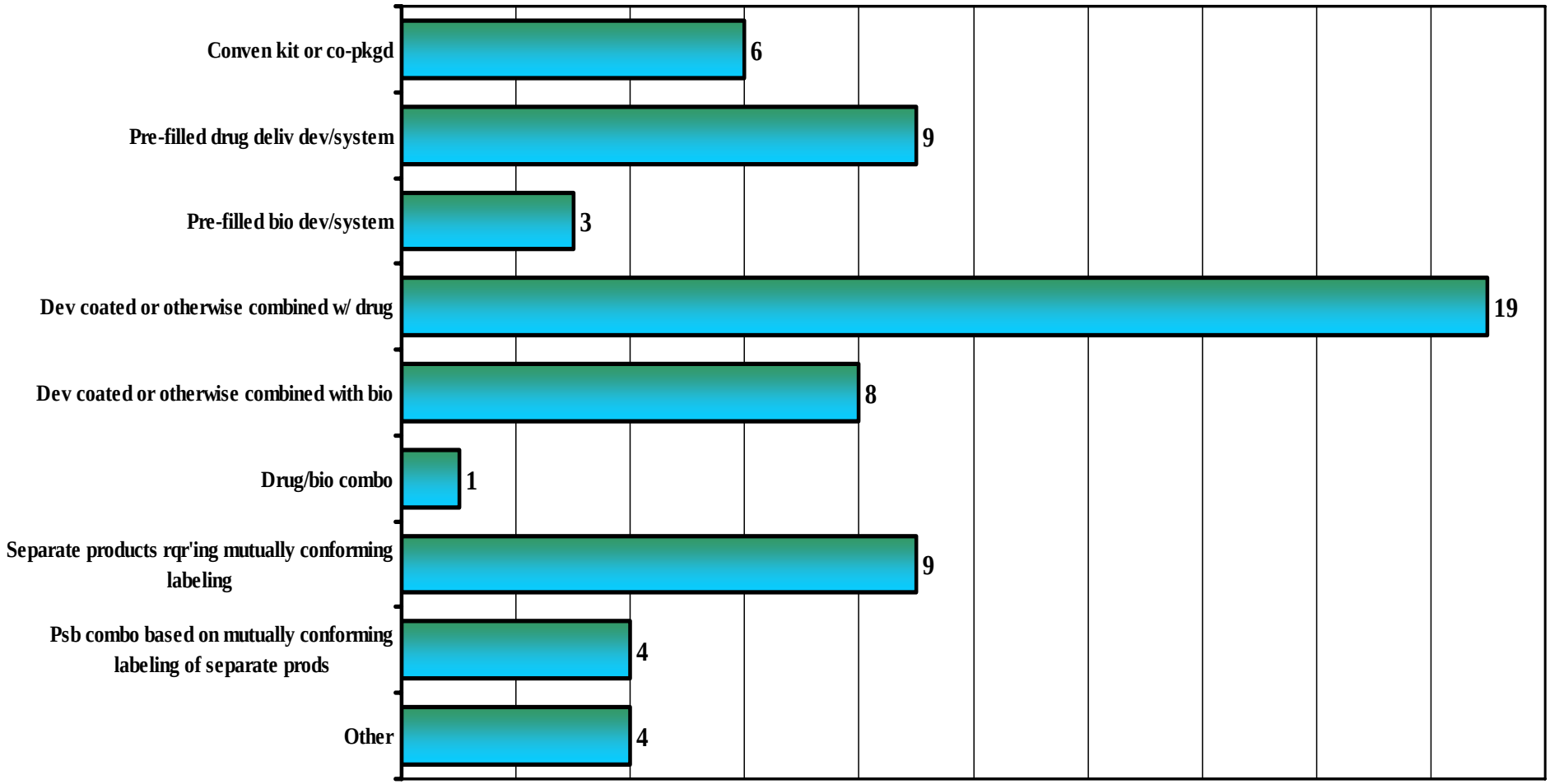
Types of combination products currently developing or marketing



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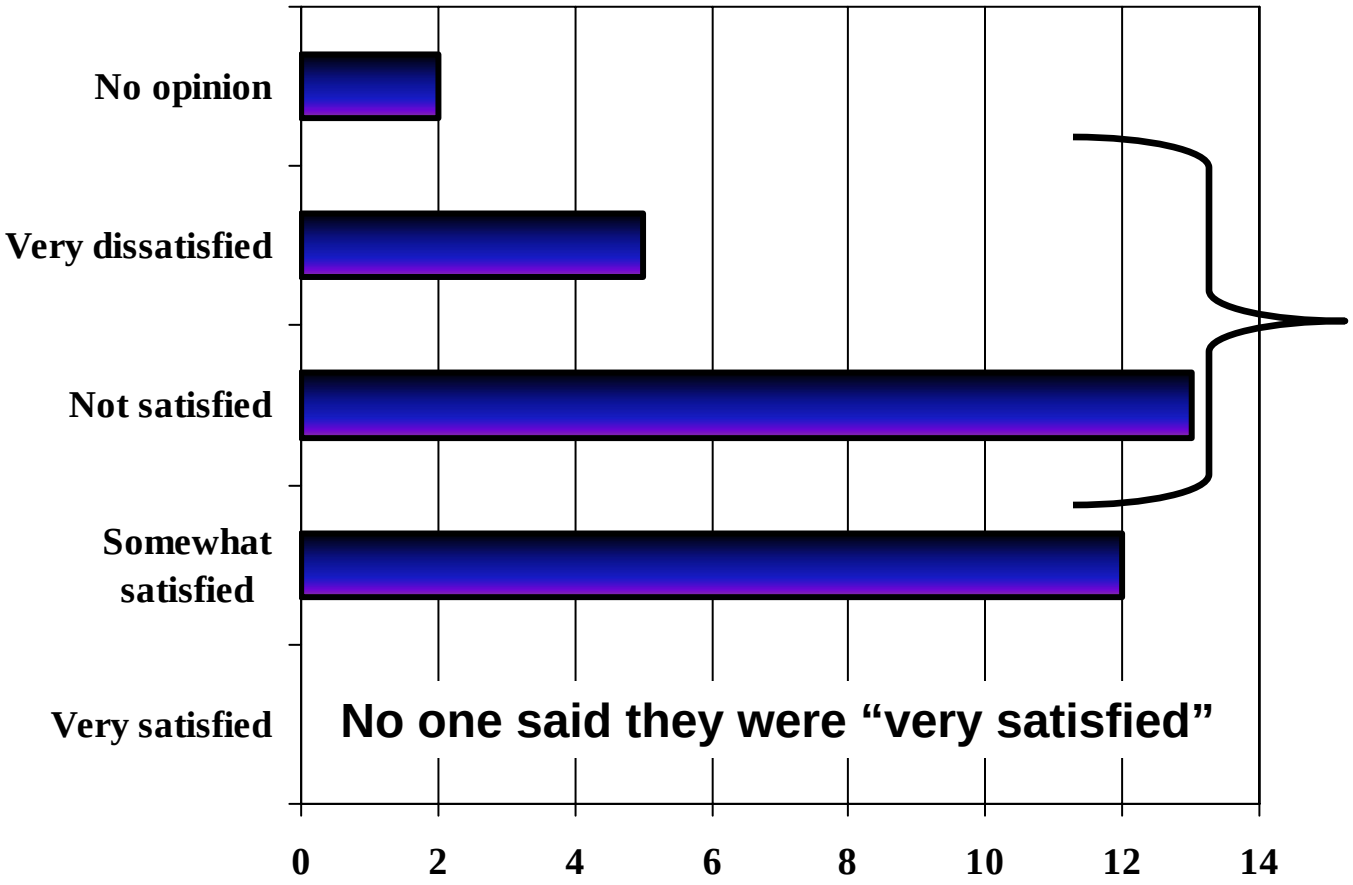
Level of satisfaction with existing guidance (FDA and non-FDA)



Over 56% indicated some level of dissatisfaction with existing guidance

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Selected Comments on Satisfaction*

Part of the problem with existing guidance documents is that they are at the 40,000 foot level, and there needs to be more detailed regulatory guidance at the 10,000 foot level.

FDA should specify in detail [applicable] pharmaceutical requirements, especially for efficacy, quality, and safety.

The commercial application of a device (which is meant to be modified and continually improved) and a drug/biologic (which is meant to stay the same) is leading to horrific change control on the device side . . . .

*Comments edited for clarity

Observations on Satisfaction

- Generally, the companies indicating that they were "Not satisfied" or "Very dissatisfied" didn't vary much by experience level or by company size
  - However, all 3 respondents who described themselves as having "No experience" were either "Not satisfied" or "Very dissatisfied"
- Only 1 of 6 respondents who were in the "premarket submission" level of development indicated any level of satisfaction
- Only 1 of 5 pharmaceutical companies indicated any level of satisfaction

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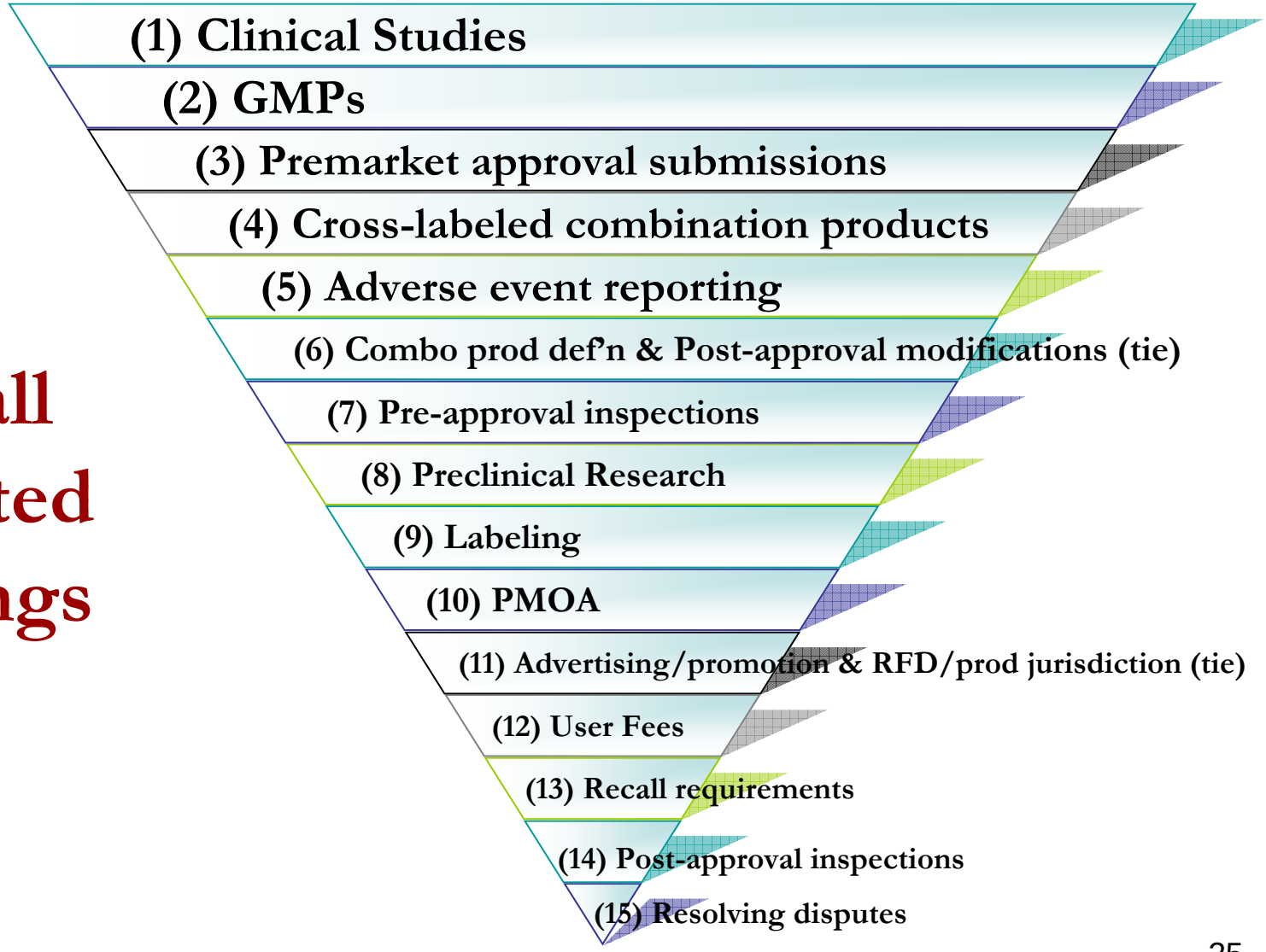
Overall Weighted Rankings



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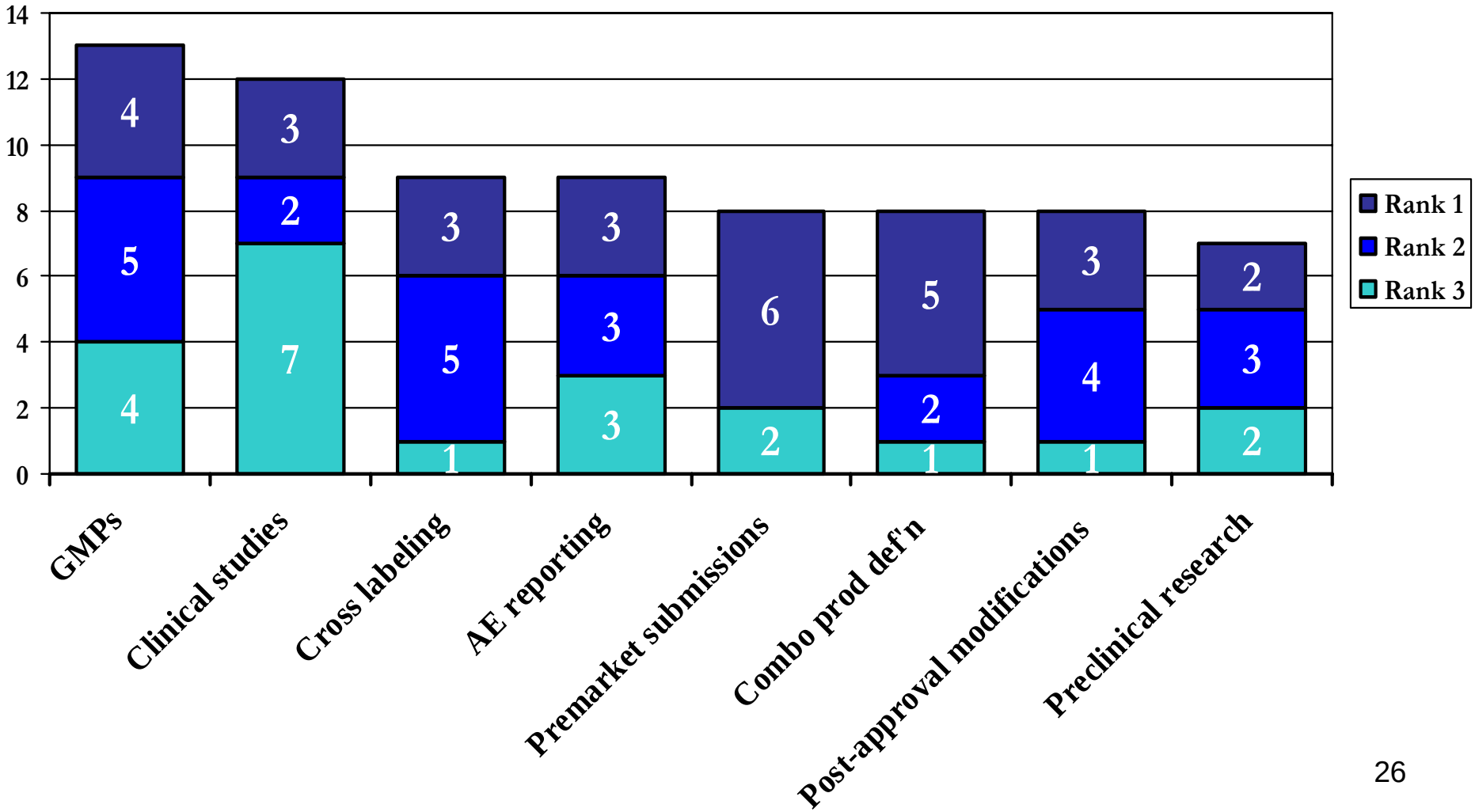
Top-Rated Guidance Topics (Not Weighted)



Observations on Topics

- GMPs
  - The only companies ranking this as #1 were self-described moderately experienced companies with less than $100 million in revenue
  - Similarly, nearly 80% of companies that ranked GMPs as 1 or 2 were "moderately experienced"
- Research
  - 20% of all respondents put research (whether as preclinical or clinical) as one of their top 3 priorities, although start-ups and companies with revenue over $1 billion were somewhat less concerned
  - Of those putting research in the top 3, only 2 were not device companies (and of these 2, it ranked 3rd)

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Observations on Topics

- Adverse event reporting
  - Not ranked highly by companies with low or no experience
  - Not surprisingly, 8 out of 9 companies ranking AE reporting as a top 3 priority was in "postmarket" stage of development
- Also not surprisingly, nearly all companies ranking post-approval modification issues as a high priority were in the "postmarket" stage
- No drug or biological company ranked "combination product definition" in their top 3 priorities, but this seems to be a concern among all types of device companies
- Concerns about cross-labeling and premarket approval submissions remained fairly consistent across company size, development stage, and experience

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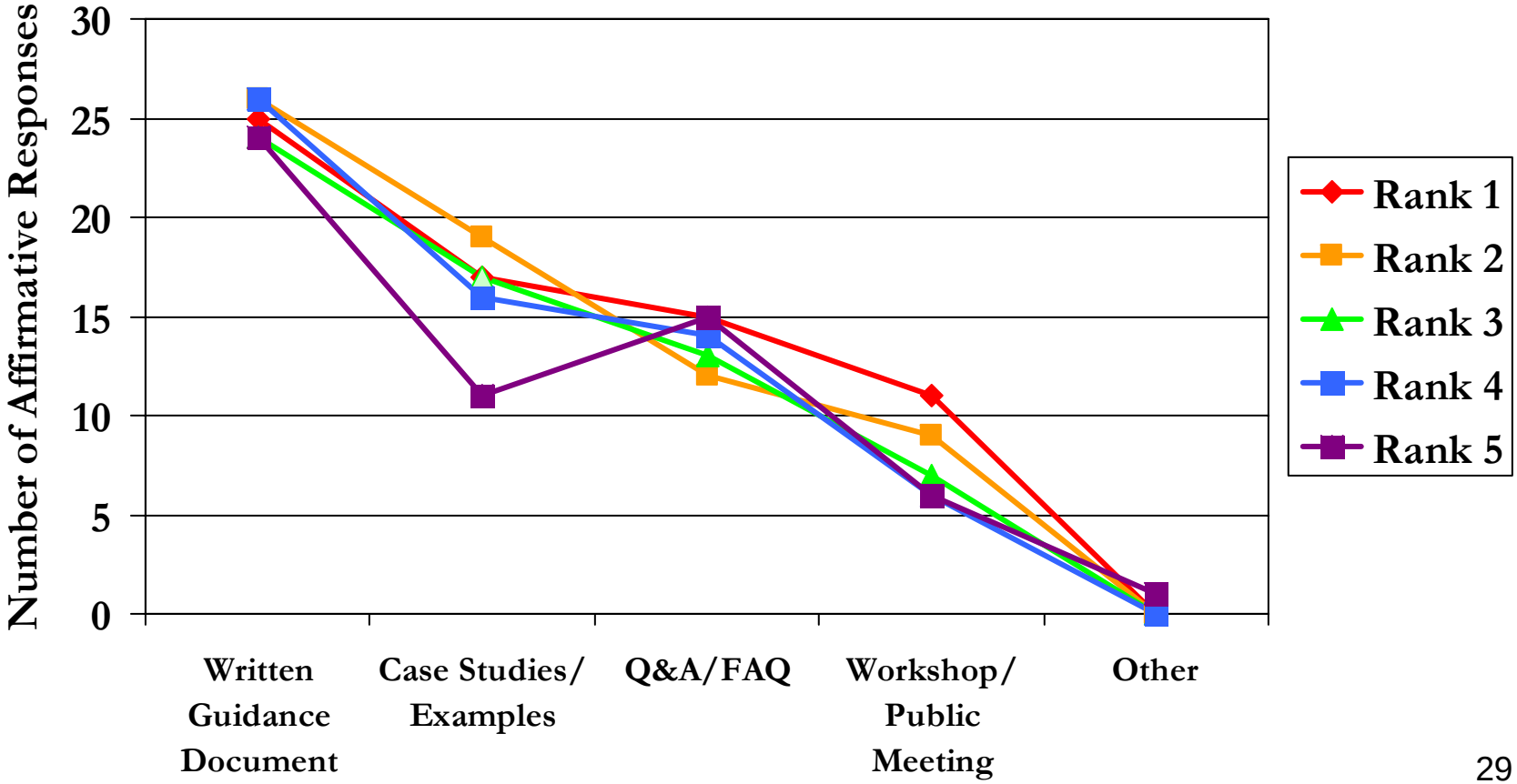
Type of Guidance Needed



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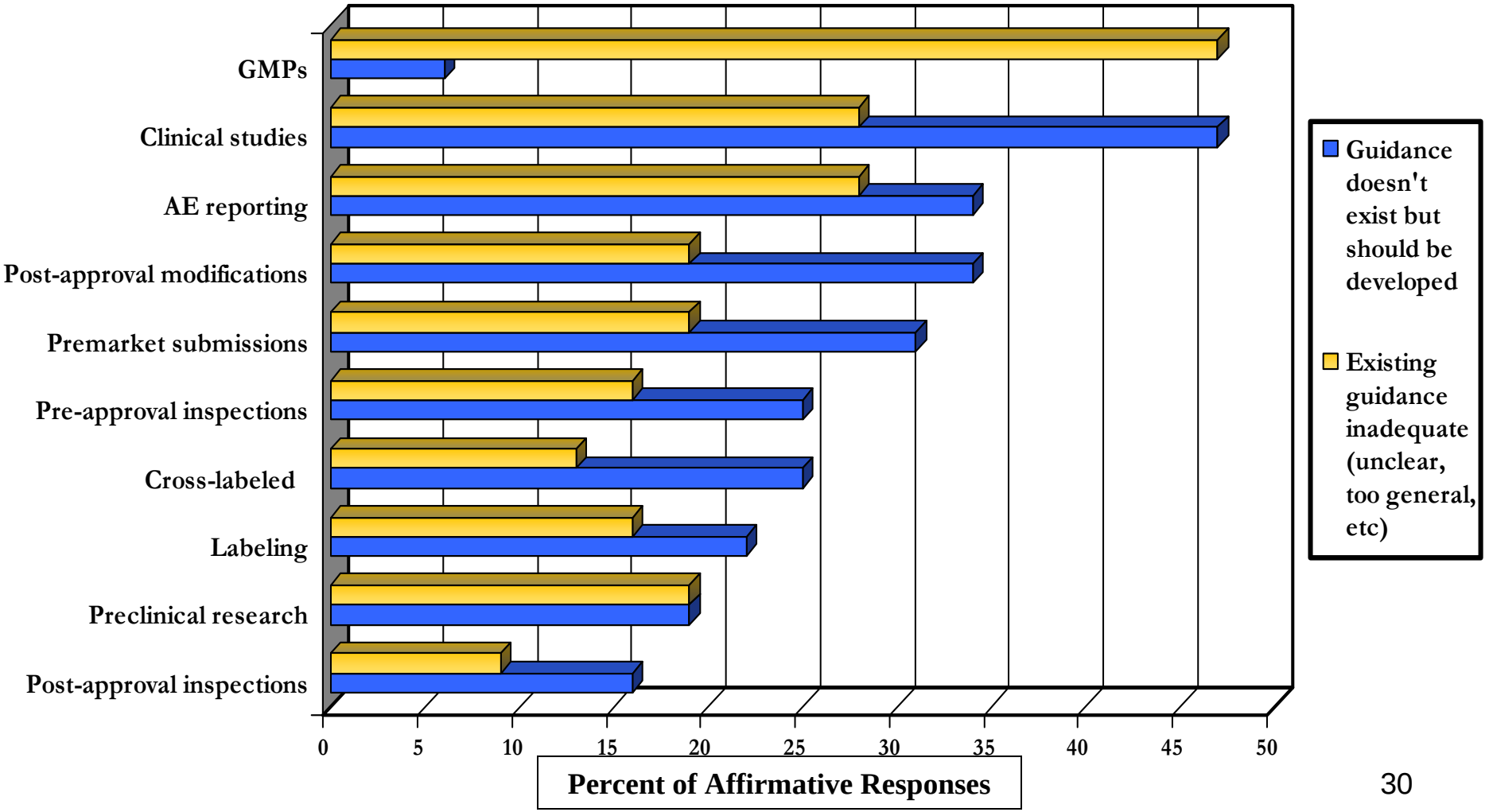
Why Guidance is Needed



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Survey says …

- Highest priority topics:
  - GMPs (which could be resolved soon)
  - Research
  - Several topics at perhaps a lower priority (AE reporting, cross-labeling, post-approval modifications, the definition of a combination product, premarket submissions)

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Survey says …

- Consensus that guidance is needed on several topics, particularly written guidance documents and, to a lesser extent, case studies/examples and FAQs
- We'll be conducting further analysis in the coming weeks as we plan for an FDA meeting

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Next Steps

1. Continued detailed analysis of survey data
2. Request FDA Meeting
3. Article in *MX* magazine
4. Open up the survey again?
    - Additional participation by pharmaceutical and biological companies

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FDA Meeting

- Request meeting with Thinh Nguyen, the new OCP director, as well as other key OCP staff
- Intended topics for discussion are:
  - Survey results
  - Potential impact on FDA policy-making agency
  - How the CPC can help
- Include a representative from: California Healthcare Institute, IMDMC, MDMA, NEMA, and RAPS

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