

World Orphan Drug Congress

Europe 2010

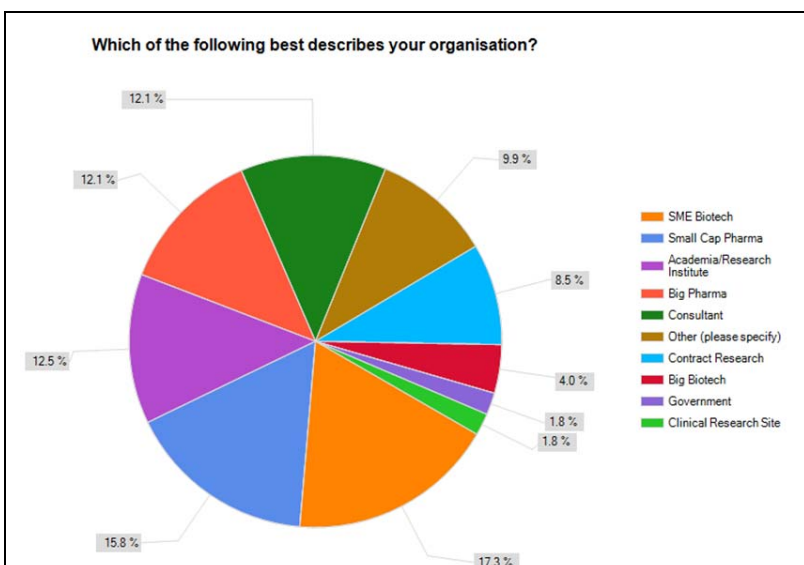
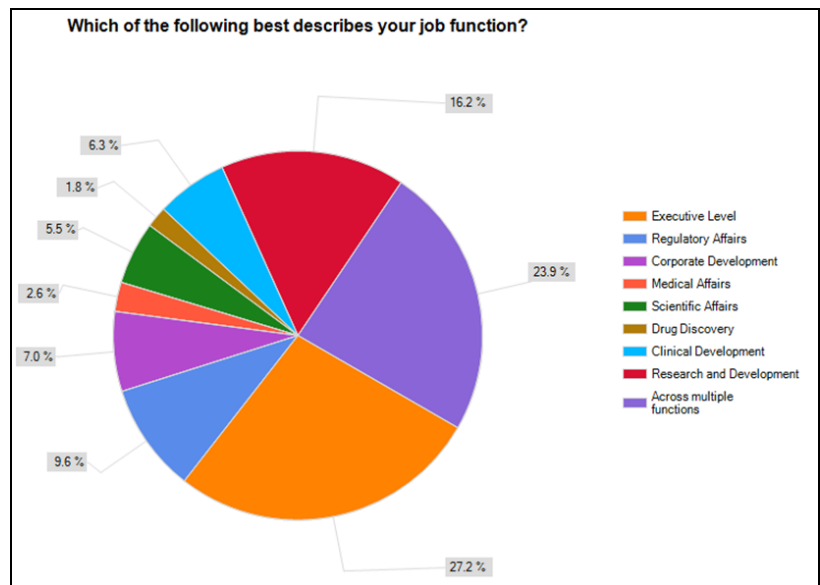
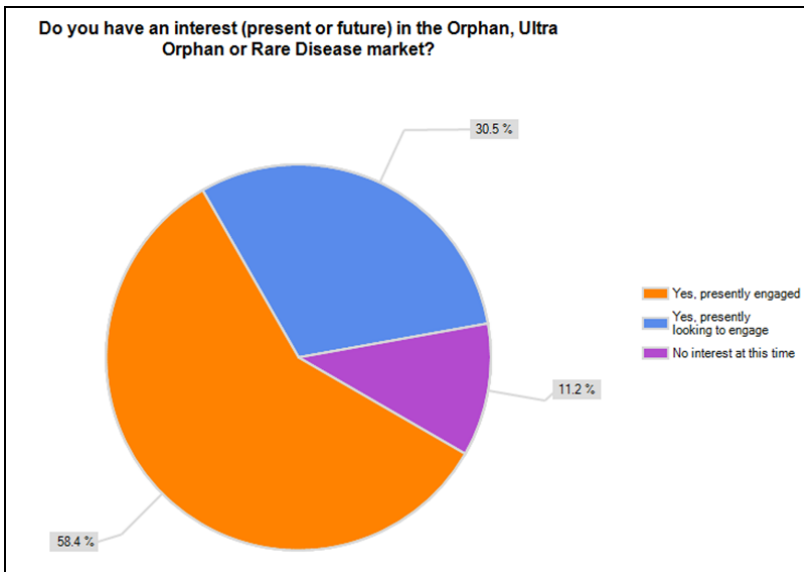
Online Survey Summary

Extract the
potential

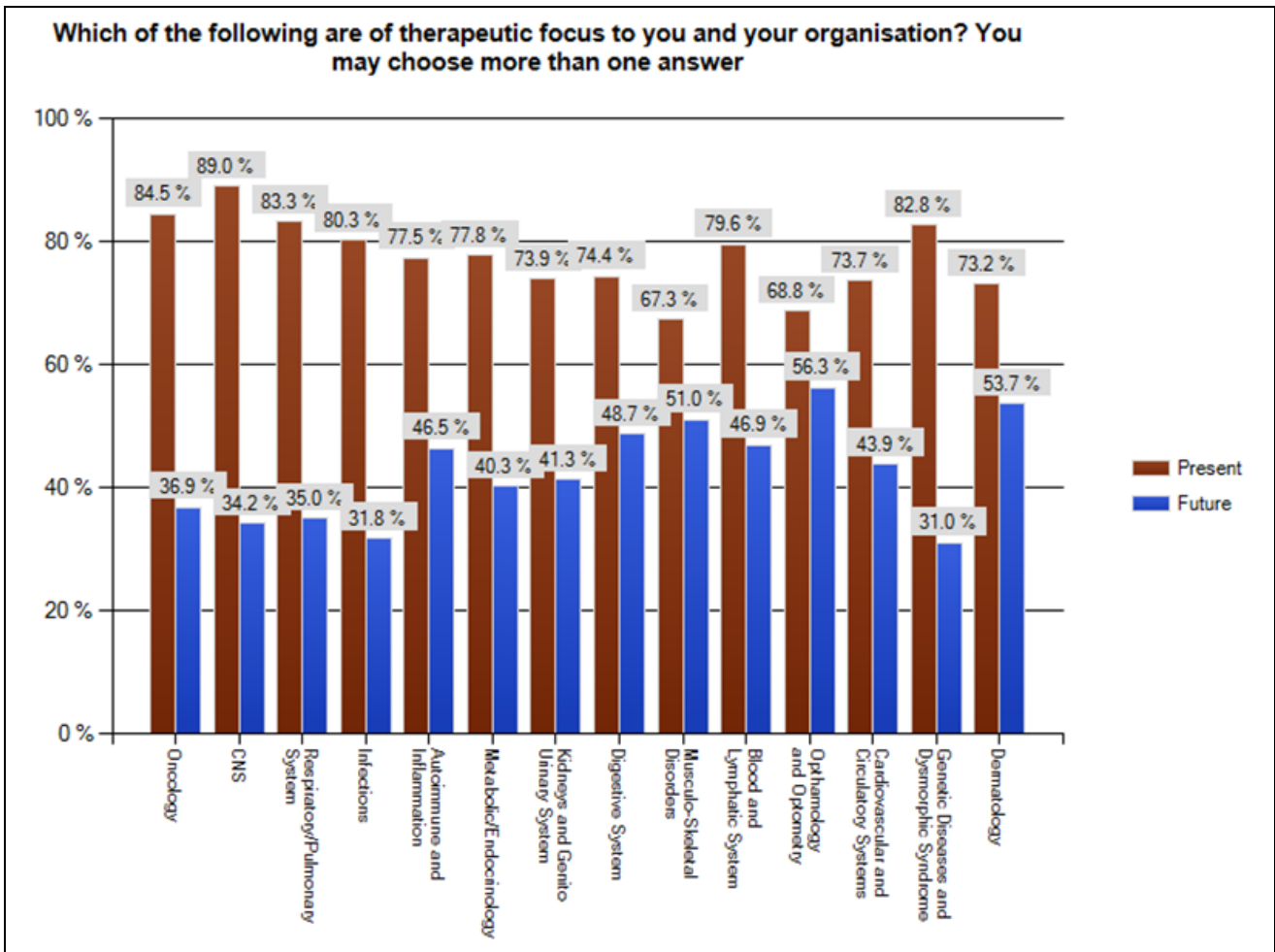


The 2010 World Orphan Drug Congress was completed by over 300 respondents from across the pharmaceutical industry. What is represented in the following document is a representational review of the survey responses provided by those survey respondents.

Q1-3 Your role and priorities



Q4.

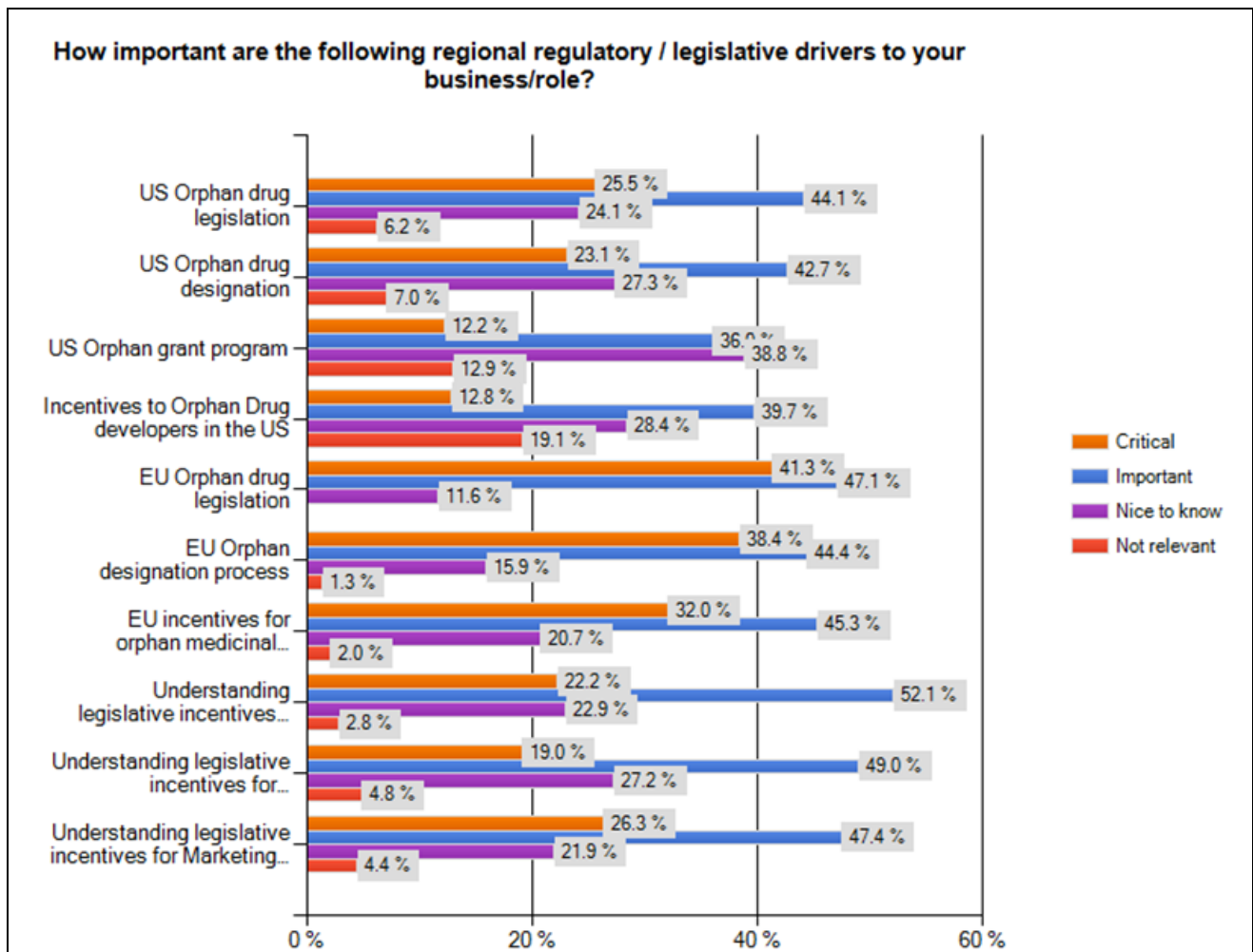


If you have selected more than 3 areas, which are the 3 most important to you right now?

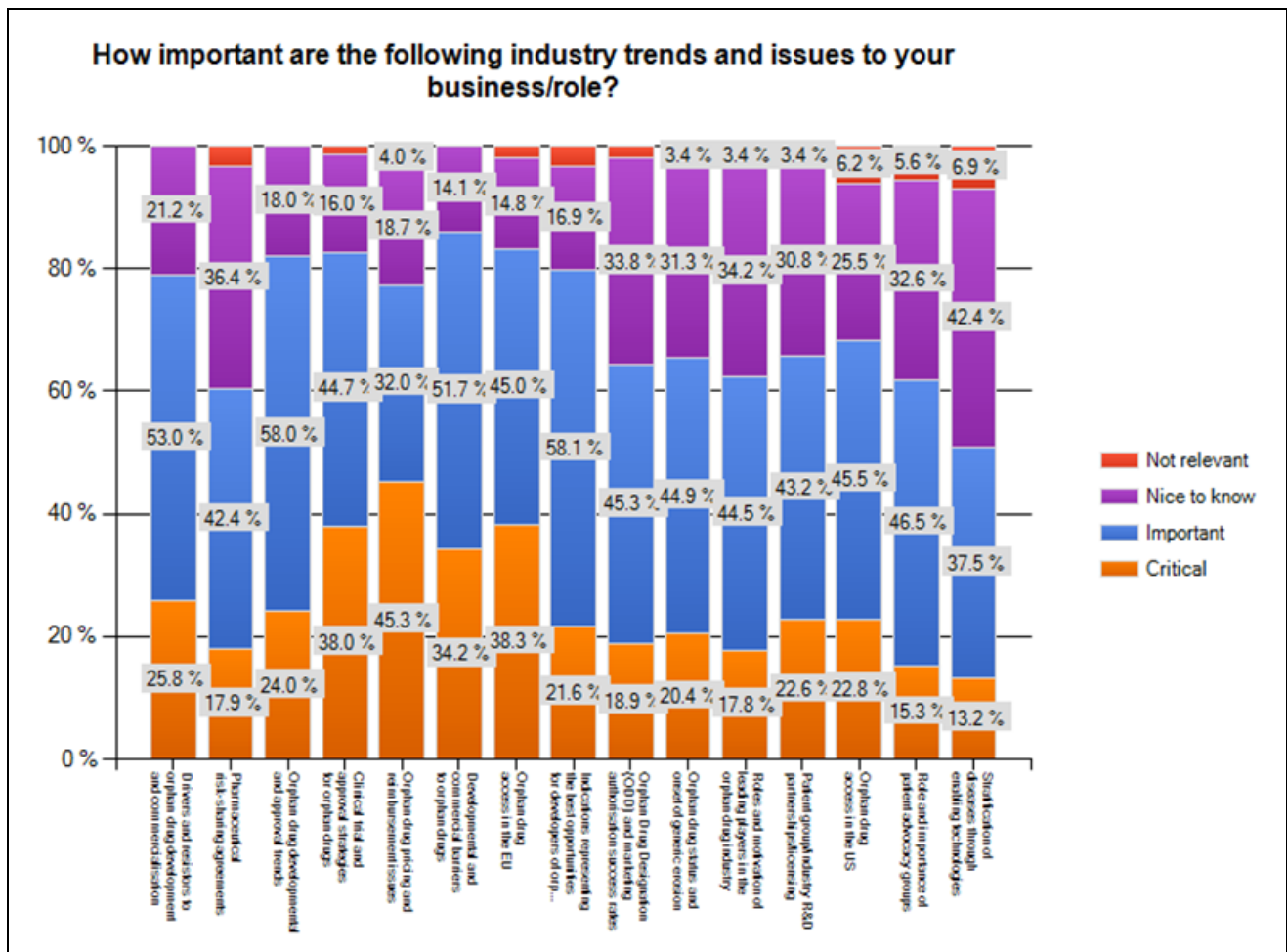
Oncology	20%
Autoimmune and Inflammation	11%
Cardiovascular and Circulatory Systems	10%
CNS	10%
Respiratory/Pulmonary System	10%
Metabolic/Endocrinology	8%
Genetic Diseases and Dysmorphic Syndrome	7%
Infections	6%
Blood and Lymphatic System	4%
Dermatology	4%
Kidneys and Genito Urinary System	3%
Ophthalmology and Optometry	3%
Digestive System	3%
Musculo-Skeletal Disorders	1%

(ie. *Oncology* was mentioned by 20% of all respondents within the Top 3 areas)

Q5.



Q6.

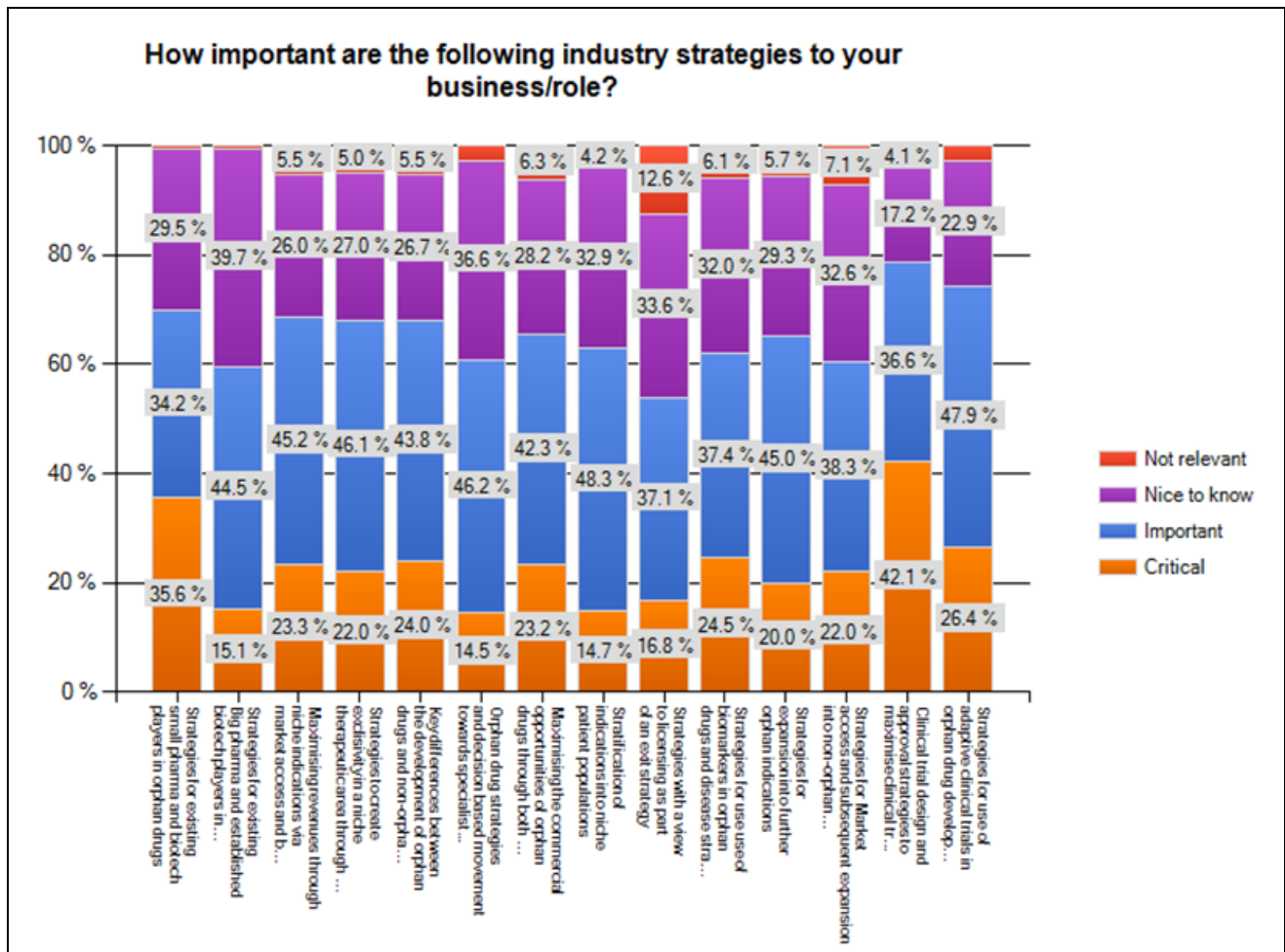


Which are the 3 most important to you right now?

Orphan drug pricing and reimbursement issues	17%
Clinical trial and approval strategies for orphan drugs	14%
Orphan drug access in the EU	10%
Orphan drug developmental and approval trends	9%
Developmental and commercial barriers to orphan drugs	9%
Drivers and resistors to orphan drug development and commercialisation	7%
Orphan drug access in the US	7%
Patient group/industry R&D partnerships/licensing	6%
Role and importance of patient advocacy groups	6%
Indications representing the best opportunities for developers of orphan drugs	5%
Stratification of diseases through enabling technologies	3%
Pharmaceutical risk-sharing agreements	2%
Roles and motivation of leading players in the orphan drug industry	2%
Orphan Drug Designation (ODD) and marketing authorisation success rates	2%
Orphan drug status and onset of generic erosion	1%

(ie. Orphan drug pricing and reimbursement issues was mentioned by 17% of all respondents within the Top 3 areas)

Q7.



Which are the 3 most important to you right now?

Clinical trial design and approval strategies to maximise clinical trial success	14%
Strategies for existing small pharma and biotech players in orphan drugs	13%
Strategies for Market access and subsequent expansion into non-orphan diseases	12%
Key differences between the development of orphan drugs and non-orphan drugs	7%
Strategies with a view to licensing as part of an exit strategy	7%
Strategies for use of adaptive clinical trials in orphan drug development	7%
Maximising the commercial opportunities of orphan drugs through both revenue expansion and protection	7%
Strategies for use of biomarkers in orphan drugs and disease stratification	6%
Maximising revenues through niche indications via market access and broadening of the product label	6%
Strategies for expansion into further orphan indications	5%
Strategies to create exclusivity in a niche therapeutic area through multiple indications	5%
Orphan drug strategies and decision based movement towards specialist indications	4%
Stratification of indications into niche patient populations	4%
Strategies for existing Big pharma and established biotech players in orphan drugs	3%

(ie. Clinical trial design and approval strategies to maximise clinical trial success was mentioned by 14% of all respondents within the Top 3 areas)