EXECUTIVE SUMMARY•Key findings

The growing interest in rare diseases•Summary
•Introduction
•What is a rare disease?
•Why target rare diseases?

Characterization of rare diseases•Summary
•Overview
•Rare diseases
•Very rare diseases
•Ultra rare diseases
•Useful resources

Orphan drug status•Summary
•Introduction
•Legislative distinctions◦US
◦Europe
◦Japan
◦Other markets

•Tax benefits
•Patient population subsetting
•Similarity
•Summary of key considerations

Choosing rare diseases to target•Summary
•Introduction
•Key issues
•Commercial potential◦Prevalence
◦Geographic distribution
◦Disease understanding
◦Available experts
◦Current treatments
◦What is similar?

•Conclusion

Preclinical development•Summary
•Introduction
•Screen or repurpose?◦Repurposed
◦Directed approaches
◦Taking advantage of orphan drug status

•Other issues◦Small molecule or biological?
◦Biological test models
◦Requirements of a clinical candidate

•Conclusions

Clinical issues•Summary
•Introduction
•Clinical trial design
•Access to patients◦Geographic distribution
◦Identifying patients

•Conclusions

Commercial considerations•Summary
•Introduction
•Identifying commercially promising opportunities◦Prevalence
◦Current treatments
◦Competitive position

•Case studies◦(Untitled sub-section)
◦Gaucher disease

•Conclusions

Conclusions•Summary
•Introduction
•Checklist to consider
•Corporate strategies
•Conclusions

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•Methodology
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