# TABLE OF CONTENTS

Chapter 1  Introduction  

**PART I  Clinical study I: an example of an n-of–one trial for a rare disease**  

Chapter 2  An n-of–one RCT for intravenous immunoglobulin G for inflammation in hereditary neuropathy with liability to pressure palsy (HNPP)  

**PART II  Stakeholders’ views on n-of-one trials for rare diseases**  

Chapter 3  Stakeholders’ views on n–of–one trials for regulatory decisions on rare diseases: a qualitative analysis of a multidisciplinary stakeholder meeting  

**PART III  Clinical study II: an example of a series of n-of-one trials for a rare disease**  

Chapter 4  Ephedrine for myasthenia gravis, neonatal myasthenia, and the congenital myasthenic syndromes  

Chapter 5  Ephedrine as add-on therapy for patients with myasthenia gravis: protocol for a series of randomised, placebo-controlled n-of-1 trials  

Chapter 6  Ephedrine treatment for autoimmune myasthenia gravis  

Chapter 7  Ephedrine add-on treatment for autoimmune myasthenia gravis: 6-month follow-up  

**PART IV  Clinical study II follow-up: acceptability of evidence from a series of n–of–one trials to healthcare regulators**  

Chapter 8  Aggregated n-of-one trials for unlicensed medicines for small populations: a case study of trialists’, patients’, and regulators’ assessments of a trial with ephedrine for myasthenia gravis
PART V  Discussion and summary  
Chapter 9  Discussion  
Chapter 10  Summary  Samenvatting  
Addenda  
Addendum A: List of abbreviations  
Addendum B: HNPP trial published Letter to the Editor  
Addendum C: HNPP trial description of analyses  
Addendum D: MEDLINE (OvidSP) search strategy  
Addendum E: EMBASE (OvidSP) search strategy  
Addendum F: CENTRAL search strategy  
Addendum G: Cochrane Neuromuscular Disease Group Specialized Register (CRS) search strategy  
Addendum H: Methods (quasi-)RCTs  
Addendum I: Ephedrine trial figure S1. Mean individual scores  
Addendum J: Ephedrine trial figure S2. Cardiovascular effects of ephedrine compared to placebo  
Acknowledgements  
About the author  
Publications