

Contents

| | |
|--|-----------|
| Prologue | ix |
| 1. Introduction | 1 |
| Part 1: Patients and Families | 15 |
| 2. Love and Loss | 17 |
| 3. In the Beginning | 35 |
| 4. Nunzio: One of a Kind | 43 |
| 5. Connie Paradiso | 52 |
| 6. A Life on the Run | 62 |
| 7. Amy's Loves | 74 |
| Part 2: Understanding Thalassemia | 85 |
| 8. The Big Alpha | 87 |
| 9. Human Globin Messages | 104 |
| 10. Finding the Genes | 117 |
| 11. The Ultimate Answers | 134 |
| 12. You Can't Go Home Again | 158 |

| | |
|---|------------|
| Part 3: The Best Medicine: Current Care and Future Goals | 183 |
| 13. The Standard of Care | 185 |
| 14. Dedicated Doctors | 197 |
| 15. Human Beta Globin Gene Therapy | 203 |
| 16. Genetic Roulette | 219 |
| 17. Modern Times | 229 |
| 18. Love Unbound | 239 |
| 19. The Last Chapter | 245 |
| Acknowledgments | 251 |
| Appendix | |
| Figures | 252 |
| Selected References | 257 |
| About the Author | 266 |
| Index | 267 |