## Contents

Contributors		i
Pre	eface	λ
1.	Challenges in progressing cell therapies to the clinic for Huntington's disease: A review of the progress made with pluripotent stem cell derived medium spiny neurons Patricia Garcia Jareño, Oliver J.M. Bartley, Sophie V. Precious, Anne E. Rosser, and Mariah J. Lelos	1
	1. Introduction	
	<ol> <li>Introduction</li> <li>Derivation of MSN-like cells from hPSCs: What has been shown in vitro and in</li> </ol>	2
	vivo?	4
	3. Evidence for functional recovery following CRT and underlying mechanisms	14
	4. Challenges	22
	5. Conclusions and authors perspectives	39
	References	41
2.	Challenges of cell therapies for retinal diseases  Monville Christelle, Morizur Lise, and Karim Ben M'Barek	49
	1. Introduction	50
	2. Retinal disorders: Focus on AMD and RP	51 54
	Human pluripotent stem cells for retinal cell therapy     Transposing bench-scale practices to industrialization	62
	5. Conclusion	66
	Funding Statement	66
	References	66
3.	Cell transplantation to repair the injured spinal cord	79
٠.	Adam Hall, Tara Fortino, Victoria Spruance, Alessia Niceforo,	
	James S. Harrop, Patricia E. Phelps, Catherine A. Priest,	
	Lyandysha V. Zholudeva, and Michael A. Lane	
		81
	<ol> <li>Introduction</li> <li>Transplantation for spinal cord repair</li> </ol>	82
	3. Pro-regenerative transplants: Bridging for spinal cord repair	83
	4. Ruilding novel neuronal pathways: Neuronal relays for repair	113

vi	Contents
----	----------

	5. Cell transplantation for neuroimmune modulation and neuroprotection	124
	6. Considerations for contraindications and adverse effects	127
	7. Closing remarks	127
	References	129
	Further reading	158
4.	Investigating cell therapies in animal models of Parkinson's and	
	Huntington's disease: Current challenges and considerations  Mariah J. Lelos	159
	1. Introduction	160
	2. Challenge 1: Identifying a good model for cell transplantation studies	162
	3. Challenge 2: Survival of xenografts in rodent models	170
	4. Challenge 3: Assessing the functional efficacy of the graft	174
	5. Challenge 4: Creating clinically relevant models	178
	6. Discussion	182
	References	183
5.	Considerations for the use of biomaterials to support cell therapy in neurodegenerative disease  Victoria H. Roberton and James B. Phillips	191
		100
	Using biomaterials to support cell therapies     Considerations for the use of his materials.	192
	<ul><li>2. Considerations for the use of biomaterials</li><li>3. Promising biomaterial and cell based therapies for neurodegenerative</li></ul>	193
	disease	200
	4. Clinical translation	201
	5. Conclusion	202
	References	203
6.	Challenges of translating a cell therapy to GMP	207
	Gerhard Bauer and Brian Fury	
	1. The history of cell and gene therapy manufacturing	208
	2. Characterization of the NSCs	220
	3. History and track record	230
	4. QC/QA at the UC Davis GMP facility	230
	References	233

7.	Considerations for clinical trial design and conduct in the evaluation of novel advanced therapeutics in neurodegenerative disease	235
	Cheney J.G. Drew and Monica Busse	
	1. Introduction	236
	2. Principles of clinical trial design	239
	3. Outcome measures in cell and gene therapy trials in neurodegenerative disease	248
	<b>4.</b> Technical and practical factors for consideration in operationalizing the intracranial delivery of cell and gene therapies	258
	5. Considerations for the inclusion of participants in trials of cell and gene	250
	therapies	262
	6. Summary	267
	References	267
8.	More than a participant in trials of cell and gene therapy: Hearing the voices of people living with neurodegenerative diseases  Emma L. Lane, Lyndsey Isaacs, and Soania Mathur	281
	1. Introduction	282
	2. Public and patient perceptions of cell and gene therapy	284
	3. The experiences of clinical trial participants with neurodegenerative diseases	287
	4. How is the voice of those living with degenerative disease engaged with?	299
	5. Factors that contribute to a meaningful patient voice	304
	6. The patient voice in clinical delivery	305
	7. Conclusion	305
	Acknowledgments	306
	The authors	306
	References	306