
Contents

OPEN Open access article

**full color
online** Supplemental
4/c image online

- **Preface 1**
Thomas Ciulla, MD, MBA, Mark E. Pennesi, MD, PhD,
Szilárd Kiss, MD and Emmett T. Cunningham, Jr, MD, PhD, MPH

- **DNA- and RNA-based Gene Therapies in
Ophthalmology 3**
**full color
online** Thomas Ciulla, MD, MBA, Mark E. Pennesi, MD, PhD,
Szilárd Kiss, MD and Emmett T. Cunningham Jr, MD, PhD, MPH

- **Expression Cassette Design and Regulation of
Transgene Expression 17**
**full color
online** Annahita Keravala, PhD and Mehdi Gasmi, PhD

- **Delivery of Genetic Information: Viral Vector
and Nonviral Vector Gene Therapies 35**
Michael Gemayel, MD, Leah C. Byrne, PhD,
Thomas Ciulla, MD, MBA and José-Alain Sahel, MD

- OPEN** ■ **Viral Vector Technologies and Strategies:
Improving on Nature 59**
Roxanne H. Croze, PhD, Melissa Kotterman, PhD,
Christian H. Burns, MS, Chris E. Schmitt, PhD,
Melissa Quezada, BS, David Schaffer, PhD, David Kirn, MD
and Peter Francis, MD, PhD

- **Manufacturing of Viral Gene Therapies 91**
**full color
online** G. Anthony Ramirez, BS and Mehdi Gasmi, PhD

- **Animals Models of Inherited Retinal Disease 113**
Ala Moshiri, MD, PhD

- **Administration of Ocular Gene Therapy 131**
David Xu, MD, M. Ali Khan, MD, Michael A. Klufas, MD
and Allen C. Ho, MD

- **Ocular Inflammation and Treatment Emergent Adverse Events in Retinal Gene Therapy 151**
 Neesurg Mehta, MD, Deborah A. Robbins, MSc
and Glenn Yiu, MD, PhD

- **Regulatory Challenges and Strategies for Ocular Gene Therapy Product Development 179**
Jim Wang, PhD, MBA

- **Value-based Medicine and Gene Therapy 195**
 Gary C. Brown, MD, MBA and Melissa M. Brown, MD,
MN, MBA