Record Nr. UNINA9910826134503321
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Titolo The clinibook : clinical gene transfer : state of the art / / edited by

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Pubbl/distr/stampa Paris, : EDP Sciences, 2012

ISBN 1-299-27682-2

2-84254-237-1

Edizione [1st ed.]

Descrizione fisica 1 online resource (589 p.)

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Disciplina 571.96

Soggetti Genetic transformation

Lingua di pubblicazione Inglese

Formato Materiale a stampa

Livello bibliografico Monografia

Note generali Description based upon print version of record.

Nota di bibliografia Includes bibliographical references and index.

Nota di contenuto pt. 1. Technologies and pre-clinical studies -- pt. 2. Clinical trials and

regulatory issues.

Sommario/riassunto This book provides a 2012 reference for state-of-the-art gene transfer technology and the different aspects of its clinical translation with a

focus on European-based initiatives. As examples of successful outcomes, recent clinical trials are presented together with Ethical, Safety and Regulatory issues, which are discussed. The broad range of various technologies is covered whether addressing direct in vivo gene transfer like with AAV, Adeno or non-viral vectors or ex-vivo

genetically engineered cells including induced pluripotent stem cells (iPS) with integrating vectors such as retrovirus. Instivirus or

(iPS) with integrating vectors such as retrovirus, lentivirus or transposon-derived systems. The critical path to clinical

implementation is covered in the second part describing currently available tools - such as molecular imaging, ex-vivo organ cultures and high-throughput technologies used for evaluation of criteria towards a go-or-no go decision to move to the clinic; in addition, utmost salient biosafety and immunotoxicology aspects are discussed. This book is

ideal for postgraduates, undergraduates, scientists, clinicians, regulators and patients' advocacy groups looking for states-of-the-art information as well as emerging prospects - including gene targeting and homologous recombination - in gene transfer intended for clinical

translation.