

# **Gene Therapy**

## **Part 2: Markets and Companies**

**By**

**Prof. K. K. Jain**  
MD, FRACS, FFPM  
**Jain PharmaBiotech**  
**Basel, Switzerland**

**November 2021**

**A Jain PharmaBiotech Report**

## AUTHOR'S BIOGRAPHY

Professor K. K. Jain is a neurologist/neurosurgeon with specialist qualifications including Fellowships of the Royal Colleges of Surgeons in Australia and Canada. He has trained, practiced and held academic positions in several countries including Switzerland, India, Iran, Germany Canada and USA. After retirement from neurosurgery, Prof. Jain remains a consultant in neurology. He is also working in the biotechnology/biopharmaceuticals industry and is a Fellow of the Faculty of Pharmaceutical Medicine of the Royal College of Physicians of UK. Currently, he is the CEO of Jain PharmaBiotech.

Prof. Jain's 492 publications include 35 books (6 as editor+ 29 as author) and 50 special reports, which have covered important areas in biotechnology, gene therapy and biopharmaceuticals, biomarkers: proteomics, molecular diagnostics, nanobiotechnology, and personalized medicine. Contributions to MedLink, an accredited continuing education program for neurologists, include 172 articles out of a total of 1250 articles by 450 authors. These articles are updated on a yearly basis. Prof. Jain's earlier books were the first in the areas covered: "Handbook of Laser Neurosurgery" (Charles C. Thomas, Springfield, Ill, 1983) and "Textbook of Hyperbaric Medicine" (1st ed in 1990 and 6th ed by Springer, 2017). Recent books include "Handbook of Nanomedicine" (Springer 2008, Chinese edition by Peking University Press 2011, 3rd ed 2017), "Textbook of Personalized Medicine" (Springer 2009; Japanese ed 2012; 2nd ed Springer 2015, 3rd ed 2021), "Handbook of Biomarkers" (Springer 2010; Chinese ed, Chemical Industry Press 2016, 2nd ed 2017), "Drug-induced Neurological Disorders", 4th ed (Springer 2021), "Handbook of Neuroprotection" (Springer 2011, 2nd ed 2019), "Applications of Biotechnology in Cardiovascular Therapeutics" (Springer 2011), "Applications of Biotechnology in Neurology" (Springer 2013), and "Applications of Biotechnology in Oncology" (Springer 2014). He has also edited 3 editions of "Drug Delivery System" (Springer 2008, 20012 and 2020) and "Applied Neurogenomics" (Springer 2015). Lectures on personalized medicine given at Kazakh National Medical University, Kazakhstan were translated into Russian and published as a book "Essentials of Personalized Medicine" (LITERRA Publishing House, Moscow, 2019). Currently, he is writing the "Handbook of Alzheimer Disease" to be published by Springer in 2022.

## ABOUT THIS REPORT

Prof. Jain wrote the first report on Gene Therapy in 1995, which was published by PJB Publication, London. This was followed by reports on Gene Therapy Vectors and Cancer Gene Therapy (1996). A report on Gene Therapy of Neurological Disorders was published by Decision Resources in 1997. In 1998, Prof. Jain wrote a Textbook of Gene Therapy, which was the first book on this subject to be translated into the Chinese language in 2000. A book on gene therapy companies was published in 2000 by John Wiley & Sons and continued to be updated online at Wiley's web site until 2003. In 2004, when the copyright reverted to Prof. Jain, it became part II of the Gene Therapy report. This report supercedes and updates all the previous books as well as reports.

**November 2021**  
**Copyright ©2021 by**

**Jain PharmaBiotech**  
**Bläsiring 7**  
**CH-4057 Basel**  
**Switzerland**

**Tel & Fax:** +4161-6924461  
**Email:** [info@pharmabiotech.ch](mailto:info@pharmabiotech.ch)  
**Web site:** <http://pharmabiotech.ch/>

All rights reserved. No part of this publication may be reproduced, stored in a retrieval system, or transmitted in any form or by any means, electronic, mechanical, photocopying, or otherwise without the prior written permission of the Publisher. This report may not be lent, resold, or otherwise traded in any manner without the consent of the Publisher. While all reasonable steps have been taken to ensure the accuracy of the information presented, the Publisher cannot accept responsibility for inadvertent errors or omissions.

# TABLE OF CONTENTS

<b>11. Markets for Gene Therapy .....</b>	<b>4</b>
<b>Introduction .....</b>	<b>4</b>
<b>Approved cell and gene therapy products .....</b>	<b>4</b>
<b>Gene therapy markets in various regions of the world.....</b>	<b>5</b>
<b>Gene therapy markets according to therapeutic areas.....</b>	<b>5</b>
Cancer gene therapy market .....	6
Markets for gene therapy of genetic disorders .....	6
<b>Markets for DNA vaccines .....</b>	<b>7</b>
DNA vaccines markets according to technologies .....	7
DNA vaccines markets according to therapeutic indications .....	8
DNA vaccines markets according to geographical areas .....	8
<b>Competing treatments .....</b>	<b>8</b>
Antisense.....	9
RNAi.....	9
Cell therapy .....	9
<b>Strategies for developing gene therapy markets .....</b>	<b>10</b>
Collaboration with pharmaceutical companies .....	10
Collaboration with companies developing cell-based therapies .....	10
Collaboration with academic gene therapy centers .....	10
Developing safer and cost-effective gene medicines .....	10
Intellectual property and commercialization of gene therapy .....	10
Overcoming obstructions to the development of gene therapy .....	11
<b>Unmet needs in gene therapy .....</b>	<b>11</b>
.....	12
<b>Promises and challenges for the development of gene therapy .....</b>	<b>12</b>
Development of gene therapy market in China .....	12
Challenges of developing gene therapy in the USA .....	13
Challenges of developing gene therapy in the European Union.....	13
<b>12. Companies involved in Gene Therapy .....</b>	<b>14</b>
<b>History of commercial development of gene therapy .....</b>	<b>14</b>
<b>Selection of companies and information .....</b>	<b>14</b>
<b>Supporting services for gene therapy .....</b>	<b>15</b>
<b>Profiles of Companies .....</b>	<b>16</b>
<b>Collaborations.....</b>	<b>257</b>
<b>13. References .....</b>	<b>264</b>

## Tables

Table 11-1: Approved cell and gene therapy products .....	4
Table 11-2: Gene therapy market according to regions/countries – 2020 to 2030 .....	5
Table 11-3: Gene therapy markets according to therapeutic areas – 2020 to 2030.....	5
Table 11-4: Cancer gene therapy market according to type of cancer - 2020 to 2030.....	6
Table 11-5: Gene therapy market for selected genetic disorders - 2020 to 2030.....	6
Table 11-6: DNA vaccines markets according to technologies - 2020 to 2030.....	7
Table 11-7: DNA vaccines markets according to therapeutic indications - 2020 to 2030.....	8
Table 11-8: DNA vaccines markets according to geographical areas - 2020 to 2030.....	8
Table 12-1: 10 major players in gene therapy .....	14
Table 12-2: Companies with supporting services for gene therapy .....	15
Table 12-3: Collaborations of gene therapy companies.....	257

## Figures

Figure 11-1: Unmet needs in gene therapy .....	12
--	----