

ANNALI

*dell'Istituto
Superiore
di Sanità*

Volume 47, No. 1, 2011

Contents

Editorial

A critical evaluation of the process of drug discovery and evaluation:
is the current approach the best possible one?

Enrico Garaci and Guido Rasi _____ **1**

Section I

New challenges in translational medicine:
transforming the advancement of science into cures

Edited by *Enrico Garaci and Patrizia Popoli* _____ **3**

Preface

Enrico Garaci and Patrizia Popoli _____ **5**

Innovative approaches to clinical development and trial design

John J. Orloff and Donald Stanski _____ **8**

The clinical trials transformation initiative (CTTI)

Alberto Grignolo _____ **14**

New perspective and new challenges in clinical trial regulation in Italy

Carlo Tomino _____ **19**

Evidence-based medicine: what it can and cannot do?

Goffredo Freddi and José Luis Roman-Pumàr _____ **22**

Integrating clinical research into clinical decision making

Mark R. Tonelli _____ **26**

The personalised medicine. A paradigm of evidence-based medicine

Dhavendra Kumar _____ **31**

The discovery and development of HIV therapy: the new challenges

Carlo Federico Perno _____ **41**

A brief history of antiretroviral therapy of HIV infection: success and challenges

Lucia Palmisano and Stefano Vella _____ **44**

Drugs and Clinical Trials in Neurodegenerative Diseases

Paolo Stanzione and Domenicantonio Tropepi _____ **49**

Need to improve clinical trials in rare neurodegenerative disorders <i>Maria Puopolo and Maurizio Pocchiari</i>	<u>55</u>
The dawn of mesoscopic approach in drug development <i>Alessandro Giuliani</i>	<u>60</u>
Translating basic research in cancer patient care <i>Marcello Maugeri Saccà and Ruggero De Maria</i>	<u>64</u>
Translational research on advanced therapies <i>Filippo Belardelli, Paola Rizza, Franca Moretti, Cintia Carella, M. Cristina Galli and Giovanni Migliaccio</i>	<u>72</u>
The role of the Istituto Superiore di Sanità (as the competent authority for phase I trials) in the translation of advanced therapies <i>Patrizia Popoli, Maria Francesca Cometa, Fulvia Fabi and Annarita Meneguz</i>	<u>79</u>
Rare diseases and orphan drugs <i>Domenica Taruscio and Claudio Frank</i>	<u>83</u>
Ricerca per le malattie rare. Perché? Non è meglio dedicarsi a malattie cardiovascolari o tumori? <i>Giuseppe Remuzzi and Arrigo Schieppati</i>	<u>94</u>
Orphan drugs assessment in the centralised procedure <i>Giuseppe Nisticò</i>	<u>98</u>
Emerging complexity in medicine: how to translate new scientific advancements into better patient care <i>Mariarita Cassese</i>	<u>100</u>
How to cope with complexity and adapt new scenarios to patient care <i>Sergio Dompé</i>	<u>104</u>
Box: Emerging complexity and possible new approaches from a patients' perspective <i>Rosaria Iardino</i>	<u>109</u>
Section II	
BOOK REVIEWS, NOTES AND COMMENTS Edited by <i>Federica Napolitani Cheyne</i>	<u>113</u>
WHO PUBLICATIONS Edited by <i>Anna Maria Rossi</i>	<u>120</u>