

List of Abbreviations
FDA Rare Disease Patient Advocacy Day
March 1, 2012

AC	Advisory committee
ACOMS	Advisory Committee Oversight and Management Staff
ADI	Acceptable daily intake
ADME	Absorption, distribution, metabolism, and excretion
AE	Adverse event
AERS	Adverse Event Reporting System
ANDA	Abbreviated New Drug Application
API	Active pharmaceutical ingredient
BA	Bioavailability
BE	Bioequivalence
BIMO	Bioresearch Monitoring (Program)
BLA	Biologics License Application
BLS	Biologics License Application Supplement
BPCA	Best Pharmaceuticals for Children Act
CBER	Center for Biologics Evaluation and Research
CDC	Centers for Disease Control and Prevention
CDER	Center for Drug Evaluation and Research
CDRH	Center for Devices and Radiological Health
CFR	Code of Federal Regulations
CFSAN	Center for Food Safety and Applied Nutrition
cGMPs	Current Good Manufacturing Practices
cGTPs	Current Good Tissue Practices
CMC	Chemistry, manufacturing and controls
CMO	Contract manufacturing organization
CNS	Central nervous system
COI	Conflict of interest
CPI	Critical Path Initiative
CRF	Case Report Form
CRO	Contract research organization
CSO	Consumer Safety Officer
CT	Clinical trial
CTD	Common Technical Document
CVM	Center for Veterinary Medicine
DDI	Division of Drug Information
DDMAC	Division of Drug Marketing, Advertising and Communications
DESI	Drug Efficacy Study Implementation
DHHS	Department of Health and Human Services
DMC	Data Monitoring Committee

DMF	Drug Master File
DNA	Deoxyribonucleic acid
DOD	Department of Defense
DSMB	Data and Safety Monitoring Board
DSP	Drug Shortage Program
DTC	Direct to consumer
eCTD	Electronic Common Technical Document
EMA	European Medicines Agency
EO	Executive order
EPA	Environmental Protection Agency
EU	European Union
EUA	Emergency Use Authorization
FDA	Food and Drug Administration
FDAAA	Food and Drug Administration Amendments Act
FDAMA	Food and Drug Administration Modernization Act
FD&C Act (FDCA)	Federal Food, Drug, and Cosmetic Act
FIH	First in human
FOIA	Freedom of Information Act
FR	Federal Register
FTC	Federal Trade Commission
FWA	Federalwide Assurance
GCPs	Good Clinical Practices
GDUFA	Generic Drug User Fee Act
GLPs	Good Laboratory Practices
GMPs	Good Manufacturing Practices
GRAS	Generally recognized as safe
GRPs	Good Review Practices
HCP	Health care provider
HCT/P	Human cells, tissues, and cellular and tissue-based products
HDE	Humanitarian Device Exemption
HED	Human equivalent dose
HIPAA	Health Insurance Portability and Accountability Act
HUD	Humanitarian Use Device
IACUC	Institutional Animal Care and Use Committee
ICF	Informed consent form
IDE	Investigational Device Exemption
IEC	Independent ethics committee
IM	Intramuscular
IND	Investigational New Drug
IOM	Institute of Medicine
IOM	Investigations Operations Manual
IP	Intraperitoneal

IP	Intellectual property
IRB	Institutional review board
IRDiRC	The International Rare Disease Research Consortium
IVD	<i>In vitro</i> diagnostic
LOAEL	Lowest observed adverse effect level
MaPPs	Manual of Policies and Procedures
MCMs	Medical countermeasures
MDA	Medical Device Amendments
MDR	Medical Device Reporting
MDUFMA	Medical Device User Fee and Modernization Act
MMA	Medicare Prescription Drug, Improvement, and Modernization Act
MOU	Memorandum of understanding
MRHD	Maximum recommended human dose
MRSD	Maximum recommended starting dose
MTD	Maximum tolerated dose
NCATS	National Center for Advancing Translational Sciences
NCE	New chemical entity
NCTR	National Center for Toxicological Research
NDA	New Drug Application
NDC	National Drug Code
NIH	National Institutes of Health
NME	New molecular entity
NOAEL	No observed adverse effect level
NOEL	No observed effect level
NSAID	Non-steroidal anti-inflammatory drug
NSRL	No significant risk level
OC	Office of the Commissioner
OCP	Office of Combination Products
OCTGT	Office of Cellular, Tissue and Gene Therapies
ODA	Orphan Drug Act
ODE	Office of Device Evaluation
OGD	Office of Generic Drugs
OIVD	Office of In Vitro Diagnostic Device Evaluation and Safety
OMPT	Office of Medical Products and Tobacco
OND	Office of New Drugs
OOPD	Office of Orphan Products Development
OPDP	Office of Prescription Drug Promotion
OPT	Office of Pediatric Therapeutics
ORA	Office of Regulatory Affairs
ORDR	Office of Rare Diseases Research
OSHI	Office of Special Health Issues
OSI	Office of Scientific Investigations
OSMP	Office of Special Medical Programs

OTC	Over the counter
PAD	Pharmacologically active dose
PD	Pharmacodynamic
PDC	Pediatric Device Consortia
PDMA	Prescription Drug Marketing Act
PDP	Product Development Protocol
PDUFA	Prescription Drug User Fee Act
PHS	Public Health Service
PI	Package insert
PI	Principal investigator
PK	Pharmacokinetic
PLA	Product License Application
PMA	Premarket Approval
PMC	Postmarketing commitment
PMN	Premarket Notification
PMR	Postmarketing requirement
PNS	Peripheral nervous system
PPI	Patient package insert
PREA	Pediatric Research Equity Act
PRO	Patient-reported outcome
QA	Quality assurance
QbD	Quality by Design
QMS	Quality Management System
QOL	Quality of life
RCT	Randomized Clinical Trial
rDNA	Recombinant DNA
RDP	Rare Diseases Program
REMS	Risk Evaluation and Mitigation Strategies
RFD	Request for Designation
RFI	Request for Information
RFP	Request for Proposal
RNA	Ribonucleic acid
RPM	Regulatory Project Manager
RTF	Refuse to file
Rx	Prescription
SAE	Serious adverse event
SAP	Scientific advisory panel
SC	Subcutaneous
SMDA	Safe Medical Devices Act
SOP	Standard Operating Procedure
SOPPs	Standard Operating Procedures and Policies
SPA	Special Protocol Assessment
TE	Therapeutic equivalence

TRND	Therapeutics for Rare and Neglected Diseases
USDA	United States Department of Agriculture
USP	United States Pharmacopeia
VAERS	Vaccine Adverse Event Reporting System
WHO	World Health Organization