

Index

- A**
Abatacept, 557, 586, 590
Absorption, 167–168
Absorption, distribution, metabolism, elimination (ADME), 361
Absorption rate-limited kinetics, 441
Accelerated degradation, 86
Acceptable Operating Ranges, 79
Acellular pertussis vaccines, 293
Actimmune®, 633
Active pharmaceutical ingredient (API), 83
Actual wholesale price (AWP), 550
Acute rejection, 544, 550
Adalimumab, 557, 585
Adaptive immune response, 285
Adaptive immune system, 281, 370
Adeno associated virus (AAV), 370
Adenosine deaminase severe combined immunodeficiency disease (ADA SCID), 370
Adenovirus (Ad), 292, 370
Adipocytes, 365
Adjuvants, 286
Ado-Trastuzumab-/Trastuzumab Emtanside, 507
Adsorption, 87, 89, 90
Adsorption chromatography, 69
Advanced therapies, 357
Advanced therapy medicinal products (ATMPs), 357
Aerosol, 479
Affinity chromatography, 70
Aflibercept, 509
Age related macular degeneration (AMD), 375
Aggregation, 83, 85, 89, 90, 141
Alefacept, 593
Alemtuzumab, 497, 538, 544
Alkaline phosphatase (AP), 44
Allergy-specific immunotherapy, 297
Alloantibodies, 538
Allograft rejection, 537
Allometric scaling, 173
 α -helix, 19, 23, 24
 α 2-Plasmin inhibitor (α 2-PI), 452
Alzheimer's disease, 357
Anakinra, 586, 606, 634, 635
Analytical techniques, 85
Anaphylactic reactions, 141, 544
Anaphylaxis, 603
Anemia, 526
Angiogenetic, 368
Animal models, 140
Anion exchange chromatography (AEX), 65
Ankylosing spondylitis (AS), 584
Anti-angiogenic antibodies, 508–509
Anti-apoptotic, 368
Antibiotic agents, 75
Antibody dependent cellular cytotoxicity (ADCC), 30, 160, 604
Antibody-dependent cellular phagocytosis (ADCP), 160
Antibody-drug conjugates (ADCs), 166, 489, 501
Antibody fragments, 151
Antibody-mediated cytotoxicity, 544
Antibody-mediated rejection (AMR), 537, 547, 548
Antibody pharmacodynamics, 151
Antibody pharmacokinetics, 166–180
Antibody structure, 151, 158–162
Anti-CD19, 371
Anti CD20 antibodies, 491–498
Anti CTLA4 antibodies, 512
Anti-drug antibodies (ADAs), 139, 159
Anti-EGFR, 502–507
Anti-EGFR-strategies, 502–507
Anti-fibrotic, 368
Antigen-presenting cells (APCs), 286
Antigen processing, 287
Anti growth factor receptor antibodies, 502–504
Anti growth factor receptor antibodies anti-EGFR2, 507–508
Anti-hGH antibodies, 446
Anti-idiotypic IgE, 544
Anti-IL-1 β , 588
Anti-IL-5, 603
Anti-IL-6 receptor, 589
Anti-IL-12/IL-23, 589, 597
Anti-IL17A, 588
Anti-integrin, 597
Antioxidants, 90, 393
Anti PD1, 512–514
Anti PD-L1 antibodies, 512–514
Antisense oligonucleotides (ASO), 309
Antithrombin, 452, 463
Anti-TNF α agents, 584
Apheresis, 384
Application of CRISPR-Cas9 in gene therapy, 340
Aptamers, 307
Arthritides, 584
Arthritis, 548
Aseptic conditions, 384
Aseptic manufacture, 361
Assays for antibodies, 143–144
Assisted reproductive technologies (ART), 429
Asthma, 584, 602
Asymmetric field flow field, 46
Atelectasis, 482
Atopic dermatitis (AD), 584, 604
Atypical hemolytic uremic syndrome, 548
Autocrine, 98, 620
Auto-fluorescence, 394
Autoimmune disease, 291, 369, 546

Autoimmune hyperthyroidism, 546
 Autologous, 359
 Autologous transfusion, 523
 Avacincaptad pegol, 308
 Avonex[®], 632

B

Bacille Calmette-Guérin (BCG), 291
 Bacterial contamination, 62
 Bacterial polysaccharides, 287
 Bacterial vector, 292
 Basiliximab, 538, 543, 549
 B cell acute lymphoblastic leukaemia, 371
 B-cell epitopes, 288
 B-cell memory, 291
 B-cell receptor (BCR), 285
 B-cells, 140, 281, 285, 366
 B-cell tolerance, 139
 Belatacept, 538
 Belimumab, 557, 592
 Benralizumab, 557, 602, 604
 Benzonase, 391
 Betaferon[®], 632
 Betaseron[®], 632
 β-Sheet, 19, 24, 25
 β-Strands, 24
 β-Turn, 20
 Bevacizumab, 509
 Big data, 196–198
 Bimekizumab, 609
 Binding assays, 52–53
 Bioassays, 52–55
 Biobanks, 195–196, 375
 Biodegradable microspheres, 99
 Biofeedback system, 99
 Bioinformatics, 196–198
 The Biologics Price Competition and Innovation Act
 of 2009, 266
 Biomarkers, 151, 201
 Biopharming, 223
 Bioreactor, 60, 389
 Biosensor, 99
 Biosensor-pump combinations, 99, 100
 Biosimilars, 260, 261, 265–274, 523
 Biotechnological production, 57
 Bispecific antibodies, 180
 Bispecific T cell enhancer, 500
 BiTE, 500
 Black-Box warning, 602
 Blastocyst, 365
 Blinatumumab, 500
 Blood-brain barrier, 168
 Blood coagulation, 451
 Blood disorder, 363
 Blotting, 43
 B-lymphocyte stimulator (BLyS), 592
 Bone/cartilage repair, 368
 Bone marrow, 365
 Bone morphogenetic protein (BMP), 374
 Bovine spongiform encephalopathy (BSE), 63
 BRCA1, 196
 Brentuximab vedotin, 502
 Brodalumab, 557, 596
 Buffer selection, 90

C

Campath, 544
 Canakinumab, 588, 606
 Canakinumab, rilonacept, 557
 Cancer vaccines, 281
 Capillary electrophoresis sodium dodecyl sulfate (CE-SDS),
 45–46
 Capillary Zone Electrophoresis (CZE), 40
 CAR T cells, 364, 607
 Cardiomyocyte, 372
 Cationic exchange chromatography (CEX), 65
 Cationic lipids, 319
 Caveolin, 319
 CD3 receptor, 540
 CD4+ epitopes, 294
 CD4+ T-cells, 140, 287
 CD8+ epitopes, 294
 CD8+ T-cells, 287
 CD19+, 364
 CD20, 546
 CD25, 538, 542
 CD28, 538, 549
 CD34+ cell, 364
 CD52, 544
 Cell and tissue transplantation, 359–364
 Cell bank, 365
 Cell harvest, 384
 Cell immortalization technologies, 359
 Cell-mediated immunity, 281, 540
 Cell migration, 382
 Cellomics, 214
 Cell-penetrating peptides (CPPs), 319
 Cell plasticity technologies, 359
 Cell surface marker, 373
 Cell therapy, 357
 Cellular-mediated rejection, 538
 Cellular, Tissue and Gene Therapies Advisory Committee, 398
 Center for Biologics Evaluation and Research (CBER), 398
 Certolizumab, 557
 Certolizumab pegol, 586
 Cetuximab, 141, 503
 Charge heterogeneity, 37–39
 Chemistry, manufacturing, and controls (CMC), 266
 Chemogenomics, 229, 230
 Chemokines (CK), 368, 619
 Chimeric antigen receptor (CAR), 607
 Chimeric antigen receptor T cells (CART-T cells), 364
 Chimeric MABs, 159
 Chimeric mice, 373
 Chimeric monoclonal antibody, 543
 Chronic idiopathic urticaria (CIU), 584, 605
 Chronic kidney disease (CKD), 444
 Chronic lymphocytic leukemia (CLL), 372, 546
 Chronic renal insufficiency (CRI), 444
 Circular dichroism (CD), 36
 Clarification, 63, 391
 Clathrin, 319
 Clearance, 169–172
 Clinical phases I–IV, 174
 CliniMACs Prodigy, 385
 Closed-loop insulin pumps, 421
 Cluster of differentiation (CD), 364, 491
 Clustered regularly interspaced short palindromic repeats
 (CRISPR), 215, 315, 372
 Collapse temperature (T_c), 93

- Colloidal aluminum salts, 300
 Combination vaccine, 291
 Combinatorial chemistry, 228
 Combined ATMP, 363
 Committee for Advanced Therapies (CAT), 398
 Committee for Medicinal Products for Human Use (CHMP), 398
 Compact SelecT, 399
 Comparative genomics, 195–196
 Complementarity determining regions (CDRs), 144, 159
 Complement-dependent cytotoxicity (CDC), 160
 Complement factors, 285
 Complement-mediated cell lysis, 544
 Conjugated antibodies, 489
 Contaminants, 73–76
 Continuous subcutaneous insulin infusion (CSII), 421
 Corifollitropin, 433
 Cost of goods, 389
 Cost of illness (COI), 257, 262
 Cost-benefit analysis (CBA), 258, 262
 Cost-effectiveness analysis (CEA), 258, 259, 262
 Co-stimulation blockade, 549
 Co-stimulatory molecules, 286
 Cost-minimization analysis (CMA), 258, 262
 Cost-utility analysis (CUA), 259, 260, 262
 CpG motifs, 306
 Creutzfeldt-Jakob disease, 437
 CRISPR-associated protein-9 nuclease (Cas9), 217, 315, 372
 CRISPR-associated proteins (Cas), 217
 CRISPR-Cas9 system, 339–341
 CRISPR-Cpf1, 217
 Critical Material Attributes (CMAs), 78
 Critical Process Parameters (CPPs), 78
 Critical Quality Attributes (CQAs), 78
 Crohn's disease (CD), 366, 596
 Crohn's Disease Activity Index (CDAI), 597
 Cryopreservation, 365
 Cryoprotectants, 91, 393
 Cryopyrin-associated periodic syndrome (CAPS), 584, 606
 Current good manufacturing practice (cGMP), 266
 CYP enzymes, 172
 Cystic fibrosis (CF), 471
 Cytokine engineering, 637–638
 Cytokine release syndrome (CRS), 371, 541, 584, 607
 Cytokines, 286, 365, 548, 619
 Cytotoxic T-lymphocyte-associated antigen 4 (CTLA-4), 549, 590
 Cytotoxic T-lymphocytes (CTLs), 285, 366
- D**
 Daclizumab, 538, 542, 545
 Daratumumab, 498–500
 Darbepoetin alfa, 522
 Deamidation, 37–39
 De-differentiation, 374
 Delivery of proteins, 95
 Denaturation, 86
 Dendritic cells (DCs), 286, 359
 Deoxyribonuclease I (DNase I), 471
 Deoxyribonucleic acid (DNA), 363
 Depth filtration, 67
 Depyrogenation, 64
 Desensitization, 539, 548
 Desensitizing agent, 547
 Design space, 78
- Diafiltration (DF), 65, 391
 Dicer, 313
 Differentiated cell, 363
 Diffuse large B-cell lymphoma, 371
 3D-cell cultures, 225, 226
 3D technologies, 379
 Dimethyl sulfoxide (DMSO), 382
 Diphtheria toxoid, 293
 Direct-to-consumer genomic tests, 206
 Discontinuous epitopes, 288
 Disease modelling, 365
 Disease-modifying antirheumatic drugs (DMARDs), 584
 Distribution, 168–169
 Disulfide bond shuffling, 30
 Disulfide bridge formation, 30
 DNA, 73
 DNA microinjection, 218, 219
 DNase treatment, 391
 DNazymes, 309
 Dornase alfa, 471
 Dosing regimen, 166
 Double-stranded DNA, 473
 Downstream processing (DSP), 57, 391
 Drisapersen, 317
 Drug development, 117, 119, 122, 131
 Druggable genome, 199
 Drug repositioning, 230
 Drug reprofiling, 230
 Drug repurposing, 230
 Drug screening, 365
 Duchenne's muscular dystrophy (DMD), 316
 Dupilumab, 557, 605
- E**
 Economic considerations, 253–262
 Ectoderm, 366
 Eculizumab, 538, 548
 Edifoligide, 312
 Efalizumab, 158, 593
 Electric dipole moments, 24
 Electrochemiluminescence, 395
 Electronic health records (EHRs), 197
 Electrophoresis, 34
 Electrospray ionization (EI), 35
 Electrostatic interactions, 28
 Elotuzumab, 498
 Elotuzumab safety, 498–500
 Elutriation, 384
 Emapticap pegol, 308
 Embryogenesis, 373
 Embryonic stem cells, 363
 Emyema, 482
 Endocrine, 98, 620
 Endoderm, 366
 Endogenous antigens, 288
 Endothelial cell, 379
 Endotoxins, 62, 75, 395
 Engineered TCR cell therapies, 371
 Enzymatic cleavage, 404
 Enzyme-Linked Immuno Sorbent Assay (ELISA), 395
 Eosinophilic granulomatosis with polyangiitis (EGPA), 584, 608
 Epigenetics, 212–213
 Epigenetic status, 373

- Epigenomics, 212–213
Epoetin alfa, 522
Epoetins, 139
Epoetin zeta, 523
Epstein-Barr virus (EBV), 547, 550
Erythropoiesis, 521
Erythropoietin (EPO), 24, 522
Erythropoietin-stimulating agents (ESA), 522
Escherichia coli (*E. coli*), 437
Etanercept, 140, 557, 586
Eteplirsen, 316
Ethics, 361
Euploid karyotype, 373
European Commission (EC), 357
European Medicines Agency (EMA), 57, 266, 363
European union (EU), 357
Eutectic temperature (T_e), 92
Evidence-based practice, 276
Excipients, 361
Exogenous antigens, 287
Exon skipping, 316, 317
Exosomes, 320, 397
Expanded bed, 72
Expression hosts, 4–6, 10, 12, 17, 18
External guide sequences, 309
Extracellular matrix, 380
Ex-vivo gene modification, 359
Ex-vivo genetic modification of cells, 357
- F**
F(ab')₂, 160
Factor IX, 458
Factor VIIa, 461
Factor VIII, 454
Factor XIII, 451, 463
Familial cold autoinflammatory syndrome (FCAS), 606
Fc-fusion protein, 458
FcRn receptor, 456
Fetal bovine serum (FBS), 374
Fibrinolysis, 451
Fibroblast growth factor-2 (FGF2), 374
Filgrastim, 20, 525, 529
Filtration, 65, 247, 391
First in human trials, 360
Fitusiran, 314
Flip-flop pharmacokinetics, 524
Flow cytometry techniques, 366
Flow Imaging Microscopy (FIM), 46
Fluorescence-activated cell sorting (FACS), 384
Fluorescence emission, 394
Fluorescence label, 394
Fluorophore, 394
Focal segmental glomerulosclerosis (FSGS), 547
Follicle-stimulating hormone, 429–435
Follitropin alfa, 429
Follitropin beta, 429
Fomivirsen, 310
Food and Drug Administration (FDA), 57, 266, 357
Forced vital capacity (FVC), 480
Foreign particle, 382
Formulation development, 83
Fourier Transformed Infrared Spectroscopy (FTIR), 36
Fragment antigen binding (Fab), 160
Fragment crystallization (Fc), 160
Fragment variable (Fv), 160
Freeze-drying, 88, 91
Fully-closed production system, 384–385
Fully human Mabs, 159
Fully integrated closed-loop delivery, 99
Functional genomics, 195–196
Functional proteomics, 199
Fusion proteins, 100
- G**
Gamma-retrovirus (γ -RV), 370
Ganirelix acetate injection, 433
G-CSF, 20
Gel-permeation chromatography, 71
Gemtuzumab ozogamicin (GO), 501
Gene therapy, 357
Gene Therapy Medicinal Product (GTMP), 363
Genetically attenuated live vaccines, 291
Genetic disorder, 363
Genome editing, 215–218, 359, 372
Genome engineering for gene therapy, 339
Genome-wide association studies (GWAS), 206
Genotoxicity, 381
Givosiran, 314
Glass transition temperature, 92
Glioblastoma, 371
Glucagon-like peptide (GLP), 400, 417
Glycans, 141
Glycation, 38
Glycobiology, 202–203
Glycomics, 202–203
Glycosylation, 29, 57, 141, 171, 203
Golimumab, 557, 587
Good manufacturing practice (GMP), 69, 384, 400
Graft-versus-host disease (GvHD), 367
Granulocyte colony-stimulating factor (G-CSF), 23, 529–530
Granulocyte-macrophage colony-stimulating factor (GM-CSF), 529
Granulopoiesis, 521
Growth hormone, 437–441, 443–446
Growth hormone binding protein (GHBP), 439–441
Growth hormone deficiency (GHD), 441, 443
Growth hormone releasing hormone (GHRH), 438
Guide RNA (gRNA), 315
Guselkumab, 557
- H**
Hairpin loops, 27
HAMA response, 144
Hammerhead and hairpin ribozymes, 311
Heart transplantation, 545
Heavy (H) chains, 158
Helical wheel, 24
Hematologic malignancies, 371
Hematopoiesis, 521
Hematopoietic growth factors, 521
Hematopoietic stem cell, 357
Hemoglobin, 370
Hemolytic uremic syndrome, 548
Hemophilia, 453
Hepatitis B surface antigen (HBsAg), 293
HER2 dimerization inhibitor (HDI), 507
Herpes virus (HPV), 370
Heterodimer, 28

- Hexamer, 405
hGH receptor (GHR), 439
Hidradenitis suppurativa (HS), 584, 609
High-affinity IgE receptor (FcεRI), 602
High molecular weight protein (HMWP) products, 419
High throughput, 397
High-throughput screening (HTS), 199, 228
High-throughput synthesis, 228
Homology-directed repair (HDR), 215, 316, 339, 340
Hoogsteen hydrogen bonds, 315
Horseradish peroxidase (HRP), 44
Host-derived proteins, 73, 76
Human anti-mouse antibodies (HAMA), 541
Human BioMolecular Atlas Program (HuBMAP), 198
Human Cell Atlas (HCA), 198
Human embryonic kidney (HEK) 293 cell line, 391
Human embryonic stem cell based product, 361
Human Genome Nomenclature Committee (HGNC), 622
Human Genome Project (HGP), 191
Human growth hormone (hGH), 437
Human Immuno-deficiency Virus (HIV) treatment, 372–373
Humanized monoclonal antibodies, 14, 159
Human leukocyte antigen (HLA), 365, 540, 548
Human leukocyte function antigen-3, 593
Human papillomavirus (HPV), 293
Human Proteome Organization (HUPO), 199
Human Proteome Project (HPP), 199
Humoral immunity, 281
Hybridoma, 1, 14, 76, 151, 191
Hydrogen bonds, 23, 28
Hydrogen-Deuterium exchange MS (HDX-MS), 36
Hydrophobic hydration, 29
Hydrophobic interaction chromatography (HIC), 65
Hydrophobicity, 25
Hypersensitivity, 506
Hypervariable sequences, 159
- I**
Idiopathic short stature (ISS), 443
IgA, 158
IgD, 158
IgE, 158, 289
IgG, 158
IgM, 158
IL-2 receptor, 542
IL-2 receptor antagonists, 544
IL-6 receptor, 548
Imetelstat, 316
Immune agonistic antibodies, 510–512
Immune complexes, 285
Immune-oncology, 151, 172
Immune oncology MAB safety, 512–514
Immune potentiators, 299–300
Immune-privileged site, 379
Immune-related adverse events (irAEs), 512
Immune-suppressant medicine, 383
Immune tolerance, 139, 371
Immunoaffinity chromatography, 70
Immunoassays, 143, 301
Immunocytochemistry, 395
Immunogenicity, 117–120, 291, 378
Immunogenicity assessment, 144
Immunoglobulins (Igs), 158, 281
Immunological memory, 286
Immunological principles, 281
Immunoreactivity, 537
Immunosuppression, 383, 537
Immunotherapies, 294, 371
Impurities, 141, 142, 392
Inactivated vaccines, 286
Inclisiran, 315
Inclusion body, 77, 78
Induced pluripotent stem cells (iPSCs), 363, 379
Infectious diseases, 281, 285, 290, 291, 341, 347, 372
Infergen®, 632
Inflammatory bowel disease (IBD), 584
Infliximab, 140, 557, 587
Inhalation technology, 97
In-line analytical testing, 397
Innate immune system, 281, 285
Inotersen, 310
Inotuzumab ozogamicin, 502
In-process control, 393
Insulin, 98, 139, 403
Insulin aspart, 408, 413
Insulin degludec, 416
Insulin detemir, 416
Insulin glargine, 415
Insulin-like growth factor, 438
Insulin production, 383
Interactome, 214
Interchangeability, 269
Interfacial stress, 89, 90
Interferon alpha (IFNα), 622
Interferons (IFN), 619
Interleukin-2 antagonists, 542
Interleukin-2 (IL2), 538
Interleukins (IL), 619
International Conference on Harmonisation (ICH), 166, 399
International nonproprietary name (INN), 270
International Society for Stem Cell Research, 398
Intracytoplasmic sperm injection (ICSI), 429
Intradermal vaccine delivery, 299
Intravenous immunoglobulin (IVIG), 548
IntronA®, 630, 632
In vitro fertilization (IVF), 429
In-vivo activity, 375
In-vivo gene modification, 359
In-vivo gene transfer, 357
Ion exchange chromatography, 33
Ipilimumab, 512
Irradiation, 367
Isoelectric focusing (IEF), 40
Isoelectric point (pI), 171, 404
Isohormones, 431
Isomerization, 37, 38
I¹³¹ Tositumumab tiuxetan, 497
Ixekizumab, 588
- J**
Janus activated kinase, 621
Juvenile idiopathic arthritis (JIA), 584
- K**
Kappaproct®, 309
Keratinocyte cell proliferation, 592
Kidney disease, 369
Kidney transplant, 541–543, 545, 549
Kineret®, 634

- L**
- Lab-on-a-chip, 199, 226
 - Late-onset neutropenia (LON), 495
 - Leachables, 64, 87
 - Lenograstim, 525, 529
 - Lentivirus (LV), 370
 - Leukemia inhibitory factor (LIF), 374
 - Leukopheresis, 384
 - Light (L) chains, 158
 - Limbal stem cell, 365
 - Lipidomics, 203
 - Lipid rafts, 491
 - Lipopolysaccharide (LPS), 75, 286
 - Liraglutide, 417
 - Live attenuated vaccines, 291–292
 - Live vectored vaccines, 291, 292
 - Lixisenatide, 417
 - Locked nucleic acids (LNAs), 318
 - Loops and turns, 25, 28
 - Lymphatic transport, 407
 - Lymph nodes, 286
 - Lymphocyte depletion, 371, 540, 546
 - Lymphotoxin, 586, 620
 - Lyoprotectants, 91
 - Lysosomal storage disease, 370
- M**
- MAB–drug interactions, 172
 - Macrophage-colony stimulating factor (M-CSF), 626
 - Macrophages, 281, 367
 - Magnesium Wasting Syndrome (MWS), 506
 - Magnetic-activated cell sorting (MACS), 384
 - Major histocompatibility complex (MHC), 540, 626
 - Mammalian cells, 4–13, 30, 31, 58–65, 72–79, 292–295
 - Managed care organizations, 240
 - Manufacturing process, 360
 - Marketing authorization application (MAA), 398
 - Marketing authorization (MA), 363
 - Mass median aerodynamic diameter (MMAD), 479
 - Mass spectrometry (MS), 33–35
 - Master cell bank (MCB), 366
 - Matrix-assisted laser desorption (MALDI), 35
 - Mechanical pumps, 98, 99
 - Medicare Benefit Policy manual, 239
 - Megakaryocytopoiesis, 530
 - Meganucleases, 215, 372
 - Membrane filtration, 67
 - Memory B- and T-cells, 286
 - Memory B-cells, 140
 - Mepolizumab, 557, 602, 603, 608
 - Mesenchymal stromal cells (MSCs), 357
 - Mesodermal lineage, 365
 - Messenger RNA (mRNA), 305
 - Metabolomics, 201, 202
 - Metabonomics, 201, 202
 - MHC class I (MHCI), 286
 - MHC class II (MHCII), 286
 - Microarrays, 199–201, 395
 - Microbiome, 204, 205
 - Microbiota, 204
 - Microfold (M) cells, 298
 - Microneedle arrays, 299
 - MicroRNA (miRNA), 309, 312, 375, 397
 - Microvesicles, 320
 - Minicircle DNA, 327
 - Mipomersen, 310
 - Miravirsen, 310
 - Molgramostim, 530
 - Monoclonal antibodies, 88, 105, 108, 110, 113–115, 118, 120, 125, 126, 139, 151–181
 - Monophosphoryl lipid A, 300
 - Mouse embryonic fibroblast, 374
 - mRNA vaccines, 294, 295
 - Muckle-Wells syndrome (MWS), 606
 - Mucociliary clearance, 471
 - Mucosal immunization, 298
 - Multiple myeloma, 367, 372
 - Multiple sclerosis (MS), 584, 609
 - Muromonab, 537, 538, 540
 - Muscle stem cell, 365
 - Mycoplasma, 62, 395
 - Myeloablative chemotherapy, 524
 - Myeloid malignancies, 372
- N**
- N-acetylgalactosamine (GalNAc), 314
 - Nano Tracking Analysis (NTA), 46
 - Nasal influenza vaccine, 299
 - Natalizumab, 557, 600, 609
 - National competent authority (NCA), 398
 - Necitumumab, 503
 - Needle-free jet injection, 299
 - Neoantigens, 296, 371
 - Neoantigen vaccines, 296, 297
 - Neonatal Fc receptor (FcRn), 181
 - Neumega®, 634
 - Neural stem cell, 365
 - Neutralizing antibodies, 140, 288, 543
 - Neutrophil extracellular traps (NETs), 475
 - Next-generation sequencing (NGS), 194–195, 207, 397
 - N-glycosylation, 439
 - Non-Hodgkin's lymphoma, 546
 - Non-homologous end-joining (NHEJ), 215, 316, 339, 340
 - Nonlinear mixed effect modeling (NONMEM), 179
 - Nonproprietary naming, 269, 270
 - Non-self, 141
 - Non-self-antigen, 383
 - Noonan syndrome, 444
 - Normal Operating Ranges (NOR), 78
 - Nuclear Magnetic Resonance (NMR), 36
 - Nucleic acid vaccines, 294–295
 - Nucleic acids, 291, 357
 - Nusinersen, 310
 - NuThrax™, 309
 - Nutrigenetics, 203, 204
 - Nutrigenomics, 203, 204
- O**
- Obinutuzumab, 496
 - Ocrelizumab, 557, 610
 - Ofatumumab, 495
 - Office of Blood Research and Review (OBRR), 398
 - Office of Tissues and Advanced Therapies (OTAT), 398
 - Office of Vaccines Research and Review (OVRR), 398
 - Olaptesed pegol, 308
 - Oligonucleotides, 305
 - Oligopotent, 359

- Omalizumab, 557, 602
2'-O-(2-methoxyethyl) oligonucleotides, 318
Omnipotent, 359
On-a-chip technology, 226
Oprelevkin, 634
Opsonization, 181
Optogenetics, 214
Oral cholera vaccine, 291
Oral polio vaccine, 291
Oral route, 96, 97
Oral vaccines, 97
Organoids, 225, 226, 380
Organ-on-chip (OOC), 226
Orthogonal, 85
Osteoblast, 365
Oxidation, 91, 141
- P**
Packaging plasmid, 389
Pancreatic cancer, 372
Panitumumab, 503
Paracrine, 98, 620
Parallel/antiparallel manner, 25
Parenteral route of administration, 95, 96
Parkinson's disease, 357
Paroxysmal nocturnal hemoglobinuria, 548
Pathogen-associated molecular patterns (PAMPs), 286
Patisiran, 314
Pattern recognition receptors (PRRs), 286
Pegaptanib, 308
Pegasys®, 637
Pegfilgrastim, 525, 529
PegIntron®, 637
Pegnivacogin, 308
Pegpleranib, 308
Pegylation, 140, 635–637
Pen devices, 421
Pen injectors, 433
Peptide epitopes, 294
Peptide nucleic acids (PNAs), 312, 318
Peptides, 291
Peripheral lymphoid organs, 286
Permeation enhancers, 168
Personalized medicine, 207, 275, 385
Pertuzumab, 507
Peyer's patches, 286
Pharmacodynamic-mediated drug disposition (PDMDD), 526
Pharmacodynamic (PD), 105–132
Pharmacoeconomics, 255–261
Pharmacogenetic biomarker, 373
Pharmacogenetics, 205–206
Pharmacogenomics, 205–206
Pharmacokinetics and pharmacodynamics (PK/PD), 151, 173, 174
Pharmacokinetics (PK), 105–132, 166, 490, 491
Pharmacy benefits management, 244
Pharmacy informatics, 198
Phenotypic stability, 366
Phosphorothioate (PS), 310
Phosphorylation, 29
Pituitary, 437–441, 443, 445
PK/PD-modeling, 119–125, 132
PK/PD relationships, 99
Plant cells, 58
Plasmapheresis, 544, 547
Plasminogen activator inhibitor-1 (PAI-1), 452
Pleiotropic properties, 368
Pluripotent, 359
Polyacrylamide gel electrophoresis (PAGE), 42–45
Polyethylene glycol (PEG), 635
Poly-ICLC (Hiltonol®), 309
Polylactic acid–polyglycolic acid (PLGA), 99
Polymerase chain reaction (PCR), 8–11, 13, 15, 17
Polysaccharide vaccines, 293
Polysorbates, 88, 90
Population PK, 172
Porcine islet, 359
Posttranslational modifications (PTM), 4, 5, 29, 30, 57, 458
Posttransplant lymphoproliferative disorder (PTLD), 546, 549
Prader-Willi syndrome, 444
Precision medicine, 207, 275
Pre-existing ADA, 143
Preexisting immunity, 292
Preformulation, 83
Pre-miRNA, 313
Preservation, 91
Primary container, 370
Primary packaging, 86
Primary sequence, 404
Primary structure, 19–23
Prions, 74
Product consistency, 389
Product half-life, 381
Production-scale cultivation, 59
Progenitor cell, 366
Progressive multifocal leukoencephalopathy (PML), 175, 495, 591
Prokaryotic, 57
Proleukin®, 634
Prophylactic vaccines, 286
Protamine, 413
Protection against oxidation, 90
Protein A, 63
Protein chemistry, 241
Protein degradation, 96
Protein fragmentation, 40–41
Protein modifications, 33, 37, 39–41, 48, 51, 54
Protein transduction, 375
Proteogenomics, 214
Proteomics, 199, 397
Protospacer adjacent motif (PAM), 340
Proven Acceptable Range (PAR), 79
Psoriasis Activity and Severity Index (PASI), 592
Psoriatic arthritis (PsA), 584
Public health legislation, 359
Pulmonary route, 97
Pulmozyme, 471
Pure red cell aplasia (PRCA), 142, 529
Purple Book, 269
Pyrogens, 75
Pyroglutamate (pyro-Glu), 38
- Q**
Quality attribute, 392
Quality by Design (QbD), 78
Quantitative polymerase chain reaction (qPCR), 395
Quaternary structure, 28

- R**
- Radioimmunotherapeutic (RIT), 161
 - Radionuclide coupled antibodies, 489
 - Ramucirumab, 509
 - Rate-controlled delivery, 98
 - Rebif[®], 632
 - Recombinant human interferon α -2b (rhIFN α -2b), 140
 - Recombinant human interferon β (rhIFN β), 140
 - Recombinant peptide vaccines, 293
 - Recombinant subunit vaccines, 293
 - Recombinant therapeutic protein, 357–360
 - Reduce immunogenicity, 147
 - Regenerative medicine, 357
 - Regulatory T-cells, 147, 289
 - Reimbursement, 248–249, 359
 - Reproductive cloning, 374
 - Reprogram the DNA, 374
 - Repulsive interactions, 28
 - Residual water content, 94
 - Reslizumab, 557, 602, 603
 - Resonance mass measurement (RMM), 46
 - Respiratory syncytial virus (RSV), 629
 - Retinal pigmented epithelial cell (RPE), 375
 - Retroviral vector, 370
 - Reversed-phase chromatography, 33
 - Revusiran, 315
 - Rheumatoid arthritis, 546, 584
 - Rheumatology, 368–369
 - Ribonucleoproteins (RNP), 316
 - Riboswitches, 307
 - Ribozymes, 309
 - RIG-I-like receptors, 286
 - Riloncept, 607
 - Risk evaluation and mitigation strategy (REMS), 550, 596
 - Rituximab, 492–494, 538, 546, 591
 - RNA interference (RNAi), 312
 - RNA-induced silencing complex (RISC), 313
 - RNA splicing, 316
 - RNase H, 310, 318
 - RNase P, 311
 - Roferon[®]A, 630, 632
 - Romiplostim, 525, 530
 - Route of administration, 95, 142, 166, 297–299, 365
- S**
- Safety testing, 360
 - Sargramostim, 530
 - Sarilumab, 557, 589
 - Scale-out, 386
 - Scale-up, 64, 391–392
 - Secondary structure, 19, 23
 - Secretory IgA response, 297
 - Secukinumab, 557, 589
 - Sheep, Dolly, 374
 - Shelf-life, 361
 - Short stature homeobox-containing gene (SHOX), 444, 445
 - Sialic acid, 30, 430
 - Sickle cell disease, 370
 - SIDT2, 319
 - Single chain Fv (scFv), 160
 - Single genetic mutation, 372
 - Single-nucleotide polymorphisms (SNPs), 194
 - Single-use systems, 72–73
 - Site-directed mutagenesis, 215, 474
 - Size exclusion chromatography, 33
 - Small interfering RNA (siRNA), 309, 312
 - Small molecule inhibitors of mitogen-activated protein kinase (MEK1/2), 374
 - Solid organ transplantation, 537
 - Somatic cell nuclear transfer (SCNT), 374
 - Somatic cell technologies, 359
 - Somatic Cell Therapy Medicinal Product (SCTMP), 363
 - Somatostatin, 438, 439
 - Stability, 83, 85, 383
 - Stem cell, 363
 - Stem cell factor (SCF), 530, 626
 - Sterilization, 62, 392
 - Storage, 244–245, 301
 - Streptokinase, 464
 - Stress conditions, 86
 - Structural genomics, 193
 - Structural proteomics, 199
 - Subcutaneous, 142, 297, 369, 441
 - Subretinal space of eye, 360
 - Subunit vaccines, 286
 - Sub-visible particles, 382
 - Suppressors of cytokine signaling (SOCS), 620
 - Surface plasmon resonance (SPR), 52, 53
 - Surrogate antibody, 176
 - Synthetic biology, 226, 227
 - Synthetic long peptide (SLP), 296
 - Synthetic peptide vaccines, 294
 - Syringeability, 88
 - Systematic evolution of ligands by exponential enrichment (SELEX), 307
 - Systemic lupus erythematosus (SLE), 584, 591
 - Systemic RNA Interference Deficiency-1, SID-1, 319
 - Systems biology, 214–215
- T**
- TAL effectors (TALEs), 217
 - Tangential flow filtration (TFF), 65, 67, 391
 - Target cell apoptosis, 544
 - T-cell epitopes, 287
 - T-cell growth factor (TCGF), 626
 - T-cell independent B-cell activation, 140
 - T-cell proliferation, 288, 538
 - T-cell receptor (TCR), 285, 371, 542
 - T-cells, 285, 359
 - TDM-1, 507
 - Terminal heterogeneity, 76
 - Tertiary structure, 28
 - T-follicular helper cells (Tfh), 289
 - Th1 cells, 289
 - Th17 cells, 289
 - Th2 cells, 289
 - T-helper cells, 17, 285, 620
 - T-helper cells type 1, 620
 - T-helper cells type 2, 620
 - Therapeutic proteins, 1–14, 16, 105–132
 - Therapeutic vaccines, 295–297
 - Thrombin, 451
 - Thrombocytopenia, 524, 531
 - Thrombolytic agents, 464
 - Thrombopoiesis, 521
 - Thrombopoietin, 530
 - Thymoglobulin, 545, 546
 - Thymus-independent antigens, 287

Tissue-derived (decellularized) scaffold, 380
Tissue engineering, 357
Tissue-type plasminogen activator (t-PA), 451
Tocilizumab, 538, 548, 557, 590
Tolerogenic vaccines, 297
Toll-like receptors (TLRs), 286, 305
Totipotent, 366
Toxicity/iRAE management, 514
Toxicogenomics, 213, 214
Toxin conjugated, 501–502
Toxins, 293
Track and traceability, 360
Transcription activator-like effector nucleases (TALEN), 215, 339, 372
Transcription factor decoys, 309
Transcriptomics, 198, 397
Transfecting packaging cell, 389
Transforming growth factor (TGF), 620, 629
Transgenic animals, 57, 218–222, 464
Transgenic plants, 222, 223
Transmissible spongiform encephalopathies (TSEs), 62
Trastuzumab, 507
Triple helix-forming oligonucleotides, 309
Tumor-associated antigens, 295–296, 364
Tumor-associated antigen vaccines, 296
Tumor infiltrating lymphocytes (TILs), 367
Tumor necrosis factor alpha (TNF α), 557
Tumor necrosis factors (TNF), 620
Turner syndrome, 444
Two-dimensional (hyphenated) techniques, 50–51
Type I antibodies, 491
Type II antibodies, 491

U

Ulcerative colitis (UC), 596
Umbilical cord tissue, 365
Unipotent, 359
Upstream processing (USP), 57, 391
U.S. Centers for Medicare & Medicaid Services (CMS), 239
Ustekinumab, 557
Uveitis (UV), 584, 610

V

Vaccination, 281, 291–297
Vaccine categories, 281
Vaccinia virus, 292
Value, 253–258, 260, 261
van der Waals interactions, 28, 29
Variable regions (V_H and V_L), 158
Vascular cell adhesion molecule-1 (VCAM-1), 600
Vedolizumab, 557, 601
Viraferon[®], 637
Viral contaminants, 64
Viral reconstitution T cell, 367
Virus filtration, 67
Virus-like particles (VLPs), 293
Virus vaccines, 288
Vitravene[®], 310
Volanesorsen, 310
Von Willebrand factor, 453, 462

W

Wegener granulomatosis, 546
Wharton's jelly, 368
Whole cell pertussis vaccine, 293
Whole-exome sequencing (WES), 194–195
Whole-genome sequencing (WGS), 194–195
Working cell bank (WCB), 366

X

Xenogeneic, 359
Xenotransplantation, 223, 225
Xeno nucleic acids (XNAs), 308

Y

Yeast, 57
 Y^{90} Ibritumumab, 497

Z

Zinc finger nucleases (ZNFs), 215, 372
Ziv-aflibercept, 509