Table of Surrogate Endpoints That Were the Basis of Drug Approval or Licensure

Downloadable Table of Surrogate Endpoints (XLS-38KB)

What is the purpose of the Surrogate Endpoint Table?

FDA's surrogate endpoint table provides valuable information for drug developers on endpoints that may be considered and discussed with FDA for individual development programs. This table also fulfills a 21st Century Cures Act requirement to publish a list of "surrogate endpoints which were the basis of approval or licensure (as applicable) of a drug or a biological product" under both accelerated and traditional approval pathways.

Section 3011 of the 21st Century Cures Act established section 507 of the Federal Food, Drug, and Cosmetic Act (FD&C Act), which mandates that the FDA publish a list of "surrogate endpoints which were the basis of approval or licensure (as applicable) of a drug or biological product" under both accelerated and traditional approval provisions. The Surrogate Endpoint Table below fulfils this legislative requirement and is intended to provide valuable information for drug developers on endpoints that may be considered and discussed with FDA for individual development programs.

According to section 507(e)(9) of the FD&C Act "[t]he term 'surrogate endpoint' means a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure, that is not itself a direct measurement of clinical benefit, and—

''(A) is known to predict clinical benefit and could be used to support traditional approval of a drug or biological product; or

''(B) is reasonably likely to predict clinical benefit and could be used to support the accelerated approval of a drug or biological product in accordance with section 506(c).''

This surrogate endpoint table includes surrogate endpoints that sponsors have used as primary efficacy clinical trial endpoints for approval of new drug applications

(NDAs) or biologics license applications (BLAs). The table also includes surrogate endpoints that may be appropriate for use as a primary efficacy clinical trial endpoint for drug or biologic approval, although they have not yet been used to support an approved NDA or BLA. We believe that this list should facilitate consideration of potential surrogate endpoints when developers are designing their drug development programs.

What are the key considerations of the surrogate endpoint table?

- The table is intended to serve as a reference guide to help inform discussion of potential surrogate endpoints with relevant Center for Biologics Evaluation and Research (CBER) or Center for Drug Evaluation and Research (CDER) review divisions, with the intended goal of facilitating product development.
- The acceptability of these surrogate endpoints for use in a particular drug or biologic development program will be determined on a case-by-case basis. It is context dependent, relying in part on the disease, studied patient population, therapeutic mechanism of action, and availability of current treatments. A particular surrogate endpoint that may be appropriate for use in a particular drug or biologic clinical development program, should not be assumed to be appropriate for use in a different program that is in a different clinical setting.
- The table does not include composite endpoints that are a combination of biomarker surrogate endpoints and clinical endpoints. Likewise, composite endpoints of biomarker surrogate endpoints and clinical outcome assessments are also not included. If a composite endpoint was composed of multiple biomarker surrogate endpoints, that information is included on the table.
- Separate adult and pediatric sections are provided. Pharmacokinetic endpoints that have supported extrapolation from adults to children are not included in the pediatric section.
- If a surrogate endpoint was previously used to support accelerated approval of a drug or biologic but subsequent confirmatory trials failed to demonstrate the expected clinical benefit, the surrogate endpoint would no longer be accepted for this use and it was not included on the table.

What are the table's limitations?

- This table reflects surrogate endpoints that have either been already used in development programs for drugs that have been approved, or surrogate endpoints that FDA has indicated acceptance of in guidances or other documents. FDA encourages development of novel surrogate endpoints, and strongly encourages sponsors to seek advice from the relevant CBER or CDER division of such novel endpoints early in development by scheduling a PDUFA VI Type C SE meeting to

discuss the use of a novel surrogate endpoint in their planned clinical trials. The acceptability of a surrogate endpoint for an individual drug or biologic development program will be determined on a case by case basis.

- The Surrogate Endpoint Table is not a replacement for discussions with appropriate CBER or CDER review divisions. Sponsors are reminded that surrogate endpoints provided in this table are intended to facilitate but not replace discussions of individual drug development programs between the sponsor and the appropriate review division.
- The table does not include surrogate endpoints that may have been accepted for past programs but are no longer acceptable as an endpoint to support registration. As scientific understanding, clinical information, and technology evolve, a previously used surrogate endpoint may no longer be considered sufficiently robust or appropriate for use in current programs.

The SE table was last updated July 23, 2018 and will be updated by CBER and CDER every 6 months to reflect current thinking as mandated by section 507 of the FD&C Act.

Footnotes

Surrogate endpoint is part of a composite of biomarker surrogate endpoints.

* Mechanism agnostic refers to cases where there are many mechanisms of action associated with a surrogate endpoint, so it is not directly related to a particular causal pathway.

§ Endpoints based on changes in tumor burden may be used for both traditional and accelerated approval depending on context of use, including factors such as disease, effect size, effect duration, residual uncertainty and benefits of other available therapy.

× The agency anticipates that this surrogate endpoint could be appropriate for use as a primary efficacy clinical trial endpoint for drug or biologic approval, although it has not yet been used to support an approved NDA or BLA.

¤ Bone mineral density is an acceptable primary endpoint for establishing efficacy for the treatment of male or glucocorticoid-induced osteoporosis after efficacy based on new morphometric vertebral fractures has been established in postmenopausal women.

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Adult Surrogate Endpoint Table

Disease or Use	Patient Population	Surrogate	Type of approval

		endpoint	appropriate for
Alpha-1-antitrypsin deficiency	Patients with congenital alpha-1 antitrypsin deficiency	Plasma alpha-1 proteinase inhibitor	Traditional
Acetylglutamate Synthase deficiency	Patients with hyperammonemia due to N-acetylglutamate synthase deficiency	Plasma ammonia	Traditional
Acromegaly	Patients with acromegaly who don't respond to or cannot undergo other standard therapies	Serum Insulin-like growth factor-I (IGF-1)	Traditional
Acromegaly	Patients with acromegaly who don't respond to or cannot undergo other standard therapies	Serum growth hormone and serum insulin-like growth factor-I (IGF-1)	Traditional
Acute Bronchospasm	Patients with acute bronchospasm associated with reversible obstructive airway disease or exercise	Forced expiratory volume in 1 second (FEV1)	Traditional
Anthrax vaccine	Patients at high risk of exposure to anthrax	Anti-protective antigen antibody response	Traditional
Asthma	Patients with asthma	Forced expiratory volume in 1 second (FEV1)	Traditional
Benign hematology	Patients with Thrombocytopenia due to immune (idiopathic) thrombocytopenia or chronic hepatitis C	Platelet count response	Traditional
Benign hematology	Patients with chronic iron overload or non-transfusion-dependent thalassemia syndromes	Serum ferritin and liver iron concentration	Accelerated
Benign hematology	Patients with anemia due to (1) chronic kidney disease, (2) chemotherapy-induced anemia, (3) zidoviduine in patients with HIV-infection.	Hematologic response and reduction in transfusion	Traditional
Benign hematology	Patients with Severe Aplastic Anemia	Hematologic response	Traditional
Benign hematology	Patients with venous thromboembolism (VTE)/pulmonary embolism	Total venous thromboembolism and all-cause death#	Traditional
Benign hematology	Patients with Acute Lymphoblastic Leukemia	Serum asparaginase	Traditional

Cancer: hematological malignancies	Patients with diffuse large B-cell lymphoma	Event-free survival (EFS) ˣ	Traditional
Cancer: hematological malignancies	Patients with chronic myeloid leukemia; hypereosinophilic syndrome/chronic eosinophilic leukemia	Major hematologic response	Accelerated/Traditional§
Cancer: hematological malignancies	Patients with acute myeloid leukemia and acute lymphoblastic leukemia	Durable complete remission rate	Accelerated/Traditional§
Cancer: hematological malignancies	Patients with acute lymphoblastic leukemia; myelodysplastic/myeloproliferative diseases; chronic myeloid leukemia	Major hematologic response and cytogenic response	Accelerated/Traditional§
Cancer: hematological malignancies	Patients with B-cell precursor acute lymphoblastic leukemia in first or second complete remission	Minimal residual disease response rate	Accelerated
Cancer: hematological malignancies	Patients with T-cell lymphoma; mantle cell lymphoma; classical hodgkin lymphoma; anaplastic large cell lymphoma and mycosis fungoides; non-hodgkin's lymphoma; multiple myeloma; chronic myeloid leukemia; acute lymphoblastic leukemia; small lymphocytic lymphoma; Waldenström's macroglobulinemia; marginal zone lymphoma	Durable objective overall response rate (ORR)	Accelerated/Traditional§
Cancer: hematological malignancies	Patients with multiple myeloma; mantle cell lymphoma; classical Hodgkin lymphoma; follicular lymphoma; diffuse large B cell lymphoma; chronic myeloid leukemia; chronic lymphocytic leukemia ; cutaneous T cell lymphoma; all other Non-Hodgkin lymphoma	Progression free survivial (PFS)	Traditional
Cancer: solid tumors	Patients with breast cancer; ovarian cancer; renal cell carcinoma; pancreatic neuroendocrine cancer; colorectal cancer; head and neck cancer; non-small cell lung cancer; melanoma; tuberous sclerosis complex; merkel cell carcinoma; basal cell carcinoma; urothelial carcinoma; cervical cancer; endometrial cancer; hepatocellular carcinoma; fallopian tube cancer	Durable objective overall response rate (ORR)	Accelerated/Traditional§

Cancer: solid tumors	Patients with breast cancer; renal cell carcinoma; pancreatic neuroendocrine tumor; soft tissue sarcoma; ovarian, fallopian tube, or primary peritoneal cancer; prostate cancer; thyroid cancer; colorectal cancer; non-small cell lung cancer; head and neck cancer; uberous sclerosis complex; merkel cell carcinoma; basal cell carcinoma; urothelial carcinoma; cervical cancer; endometrial cancer; hepatocellular carcinoma; fallopian tube cancer	Progression free survivial (PFS)	Accelerated/Traditional§
Cancer: solid tumors	Patients with surgically resected Dukes' C colon cancer, melanoma, renal cell cancer or breast cancer	Disease-free survival (DFS)	Accelerated/Traditional§
Cancer: solid tumors	Patients with breast cancer; neuroblastoma	Event-free survival (EFS) ×	Accelerated/Traditional§
Cancer: solid tumors	Patients with breast cancer	Pathological complete response	Accelerated
Cancer: solid tumors	Patients with nonmetastatic castrate-resistant prostate cancer	Metastasis-free survival	Accelerated/Traditional§
Chronic kidney disease	Patients with chronic kidney disease secondary to multiple etiologies	Estimated glomerular filtration rate ×	Traditional
Chronic kidney disease	Patients with chronic kidney disease secondary to multiple etiologies	Serum creatinine ×	Traditional
Chronic obstructive pulmonary disease (COPD)	Patients with COPD	Forced expiratory volume in 1 second (FEV1)	Traditional
Cushing's disease	Patients with Cushing's disease	Urine free cortisol	Traditional
Cushing's syndrome	Patients with endogenous Cushing's syndrome	Urine free cortisol ×	Traditional
Cystic fibrosis	Patients with cystic fibrosis	Forced expiratory volume in 1 second (FEV1)	Traditional
Cystinuria	Patients with cystinuria	Urinary cystine	Traditional

Cytomegalovirus (CMV)	CMV seropositive and hematopoietic transplant recipients requiring prophylaxis	Plasma CMV-DNA exceeding threshold for starting treatment	Traditional
Diphtheria vaccine	Patients to be immunized against diphtheria	Diphtheria antitoxoid antibody response	Traditional
Duchenne muscular dystrophy (DMD)	Patients with DMD who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping	Skeletal muscle dystrophin	Accelerated
Exocrine pancreatic insufficiency	Patients with exocrine pancreatic insufficiency due to cystic fibrosis, chronic pancreatitis, pancreatectomy, or other conditions	Fecal coefficient of fat absorption	Traditional
Female hypogonadotropic hypogonadism	Infertile women with hypogonadotropic hypogonadism	Follicle size, serum estradiol and progesterone#	Traditional
First aid antiseptic; Health care antiseptic; Consumer antiseptic	General public, consumers, and health care professionals	Bacterial count	Traditional and Monograph
Gout	Patients with gout	Serum uric acid	Traditional
Heart failure	Patients with acute heart failure	Blood pressure	Traditional
Hepatitis A (Hep A) vaccine	Patients to be immunized against Hep A	Anti-Hep A virus antibody concentration	Traditional
Hepatitis B (Hep B) vaccine	Patients to be immunized against Hep B	Anti-Hep B virus antibody concentration	Traditional
Hepatitis B Virus (HBV)	Patients with HBV infection with or without cirrhosis	Undetectable serum HBV-DNA	Traditional
Hepatitis C Virus (HCV)	Patients with HCV infection with or without cirrhosis	Sustained viral response (HCV-RNA)	Traditional
Hepatorenal syndrome	Patients with hepatorenal syndrome type 1	Serum creatinine ×	Traditional
Homozygous sitosterolemia (phytosterolemia)	Patients with homozygous sitosterolemia (phytosterolemia)	Plasma sitosterol and campesterol	Traditional
Human Immunodeficiency	Patients with HIV-1	Undetectable plasma HIV RNA	Traditional

Virus-1 (HIV-1)			
Human Immunodeficiency Virus-1 (HIV-1)	Patients at high risk of sexually acquired HIV-1	Serum HIV antibody concentration	Traditional
Human Immunodeficiency Virus-1 (HIV-1)	Highly treatment-experienced HIV-1 patients	Greater than 0.5 log reduction in plasma HIV RNA	Traditional
Hypercholesterolemia	Patients with heterozygous familial and nonfamilial hypercholesterolemia	Serum LDL-C ×	Traditional
Hypercholesterolemia	Patients with homozygous familial hypercholesterolemia	Serum LDL-C	Traditional
Hyperkalemia	Patients with hyperkalemia	Serum potassium	Traditional
Hyperphosphatemia	Dialysis patients with hyperphosphatemia	Serum phosphate	Traditional
Hypertension	Patients with hypertension	Blood pressure	Traditional
Hypertriglyceridemia	Patients with severe hypertriglyceridemia	Serum triglycerides	Traditional
Hypokalemia	Patients with hypokalemia	Serum potassium	Traditional
Hyponatremia	Patients with hypervolemic and euvolemic hyponatremia	Serum sodium	Traditional
Hypotension	Patients with distributive shock	Blood pressure	Traditional
Hypotension	Patients with symptomatic orthostatic hypotension	Blood pressure	Accelerated
Hypothyroidism	Patients with hypothyroidism	Serum thyroid-stimulating hormone (TSH)	Traditional

Idiopathic pulmonary fibrosis	Patients with idiopathic pulmonary fibrosis	Forced vital capacity (FVC)	Traditional
Influenza vaccine	Patients to be immunized against influenza	Hemagglutination inhibition antibody response	Accelerated
Interoperative hemorrhage	Patients who require reduction of blood pressure to reduce bleeding during surgery	Blood pressure	Traditional
Japanese encephalitis vaccine	Patients to be immunized against Japanese encephalitis	Neutralizing antibody response	Traditional
Lipodystrophy	Patients with congenital or acquired generalized lipodystrophy	Serum hemoglobin A1C, fasting glucose and triglycerides	Traditional
Male hypogonadotropic hypogonadism	Men with selected cases of hypogonadotropic hypogonadism	Sperm count	Traditional
Meningococcal vaccine	Patients to be immunized against meningococcal meningitis	Serum bactericidal antibody response	Traditional
Nephropathic cystinosis	Patients with nephropathic cystinosis	White blood cell cystine and serum creatinine#	Traditional
Nonalcoholic steatohepatitis (NASH)	Precirrhotic NASH patients with liver fibrosis	Histopathologic findings of either 1) resolution of steatohepatitis with no worsening of fibrosis OR 2) improvement of fibrosis with no worsening of steatohepatitis OR 3) Both#	Accelerated
Opioid dependence	Patients with opioid dependence	Urine toxicology test for opioids	Traditional
Osteoporosis	Postmenopausal women with osteoporosis	New morphometric vertebral fractures	Traditional
Osteoporosis	Patients with glucocorticoid induced osteoporosis	Bone mineral density¤	Traditional
Osteoporosis	Men with osteoporosis	Bone mineral density¤	Traditional

Paget's disease	Patients with Paget's disease	Serum alkaline phosphatase	Traditional
Peri-implantitis	Patients with peri-implantitis	Probing pocket depth ×	Traditional
Periodontitis	Patients with chronic periodontitis with a mean probing pocket depth of greater than 5mm	Probing pocket depth	Traditional
Phenylketonuria	Patients with hyperphenylalaninemia due to tetrahydrobiopterin-responsive phenylketonuria	Plasma phenylalanine	Traditional
Pneumonia vaccine	Patients (≥ 50 years of age) to be immunized against pneumonia and invasive disease	Opsonophagocytic antibody response	Accelerated
Polio vaccine	Patients to be immunized against polio	Neutralizing antibody response	Traditional
Polycystic kidney disease	Patients with autosomal dominant polycystic kidney disease with or without associated polycystic liver disease	Total kidney volume ×	Accelerated
Preterm birth	Women with a singleton pregnancy who have a history of singleton spontaneous preterm birth	Delivery prior to 37 weeks gestation×	Accelerated
Primary biliary cholangitis	Patients with primary biliary cholangitis	Serum alkaline phosphatase and bilirubin#	Accelerated
Primary glomerular disease associated with nephrotic syndrome	Patients with primary glomerular disease associated with nephrotic syndrome	Proteinuria (urinary protein/creatinine ratio) ×	Accelerated
Primary hyperparathyroidism	Patients with hypercalcemia due to primary hyperparathyroidism	Serum calcium	Traditional
Primary immunoglobulin A nephropathy	Patients with primary IgA Nephropathy	Proteinurea (urinary protein/ creatinine ratio) ×	Accelerated
Pulmonary multi-drug resistant tuberculosis	Patients with pulmonary multi-drug resistant tuberculosis	Time to sputum culture conversion to negative	Accelerated
Pulmonary tuberculosis	Patients with active or latent pulmonary tuberculosis	Time to sputum culture conversion to negative	Accelerated

Rabies immune globulin	Patients with suspected exposure to a rabid animal	Rabies neutralizing activity and antibody response	Traditional
Secondary hyperparathyroidism	Patients with secondary hyperparathyroidism associated with chronic kidney disease	Serum intact parathyroid hormone (iPTH)	Traditional
Supportive cancer care	Patients with delayed methotrexate clearance due to impaired renal function	Plasma methotrexate	Traditional
Supportive cancer care	Patients with leukemia, lymphoma, and solid tumor malignancies who are receiving anti-cancer therapy expected to result in tumor lysis and subsequent elevation of uric acid.	Serum uric acid	Traditional
Supportive cancer care	Patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs	Duration of severe neutropenia	Traditional
Testosterone deficiency	Men with primary or hypogonadotropic hypogonadism	Serum testosterone	Traditional
Tetanus vaccine	Patients to be immunized against tetanus	Tetanus antitoxoid antibody response	Traditional
Tobacco dependence	Cigarette smokers	Exhaled carbon monoxide	Traditional
Type 1 diabetes mellitus	Patients with type 1 diabetes mellitus	Serum hemoglobin A1C	Traditional
Type 1 Gaucher disease	Patients with Type 1 Gaucher disease	Spleen volume, liver volume, hemoglobin and platelet count#	Traditional
Type 2 diabetes mellitus	Patients with type 2 diabetes mellitus	Serum hemoglobin A1C	Traditional
X-linked hypophosphatemia	Patients with X-linked hypophosphatemia	Serum phosphate	Traditional
Yellow fever vaccine	Patients at risk of exposure to yellow fever	Neutralizing antibody response	Traditional

Pediatric Surrogate Endpoint Table

Disease or Use	Patient Population	Surrogate endpoint	Type of approval appropriate for	Drug mechanism action

Acetylglutamate Synthase deficiency	Patients with hyperammonemia due to N-acetylglutamate synthase deficiency	Plasma ammonia	Traditional	Carbamoyl Phosphate Synthetase activator
Acromegaly	Patients with acromegaly who don't respond to or cannot undergo other standard therapies	Serum Insulin-like growth factor-I (IGF-1)	Traditional	Growth horm receptor antagonist
Acute bronchospasm	Patients with acute bronchospasm associated with reversible obstructive airway disease or exercise	Forced expiratory volume in 1 second (FEV_1)	Traditional	Beta-2 adren agonist
Asthma	Patients with asthma	Forced expiratory volume in 1 second (FEV_1)	Traditional	Corticostero Beta-2 adren agonist; Anticholinerg
Benign hematology	Patients with thrombocytopenia due to immune (idiopathic) thrombocytopenia or chronic hepatitis C	Platelet count	Traditional	Thrombopoi receptor ago
Benign hematology	Patients with chronic iron overload or non-transfusion-dependent thalassemia syndromes	Serum ferritin and liver iron concentration	Accelerated/Traditional§	Iron chelator
Benign hematology	Patients with severe aplastic anemia	Hematologic response	Traditional	Thrombopoi receptor ago
Benign hematology	Patients with venous thromboembolism (VTE)/pulmonary embolism	Total VTE and all-cause death	Traditional	Anticoagulat
Cancer: hematological malignancies	Patients with acute lymphoblastic leukemia	Durable objective overall response rate (ORR)	Accelerated/Traditional§	Mechanism agnostic*
Cancer: hematological malignancies	Patients with acute lymphoblastic leukemia	Event Free Survival (EFS)	Accelerated/Traditional§	Mechanism agnostic*
Cancer: hematological	Patients with chronic	Major	Accelerated/Traditional§	Mechanism

malignancies	myeloid leukemia	hematologic and cytogenic response		agnostic*
Cancer: hematological malignancies	Patients with Acute Lymphoblastic Leukemia	Serum Asparaginase	Traditional	Asparagine-specific enzy
Cancer: solid tumors	Patients with tuberous sclerosis complex with subependymal giant cell astrocytoma	Durable objective overall response rate (ORR)	Accelerated	Kinase inhib
Cancer: solid tumors	Patients with merkel cell carcinoma	Durable objective overall response rate (ORR)	Accelerated	Programmed death ligand (PD-L1) bloc antibody
Cancer: solid tumors	Patients with metastatic melanoma	Durable objective overall response rate (ORR)	Accelerated	Mechanism agnostic*
Cancer: solid tumors	Patients with metastatic melanoma	Progression Free Survival (PFS)	Accelerated	Mechanism agnostic*
Chagas disease	Patients with Chagas disease	Immunoglobulin G antibody negative against the recombinant antigens of *T. cruzi*	Accelerated	Antimicrobia
Cystic fibrosis	Patients with cystic fibrosis	Forced expiratory volume in 1 second (FEV_1)	Traditional	Cystic fibros transmembr conductance regulator potentiator
Cystinuria	Patients with cystinuria	Urinary cystine	Traditional	Reducing ar complexing
Cytomegalovirus (CMV)	CMV seropositive and hemotopoietic transplant recipients requiring prophylaxis	Plasma CMV-DNA exceeding threshold for starting treatment	Traditional	Antiviral
Diphtheria vaccine	Patients to be	Diphtheria	Traditional	Neutralizing

	immunized against diphtheria	antitoxoid antibody		antibody
Duchenne muscular dystrophy (DMD)	Patients with DMD who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping	Skeletal muscle dystrophin	Accelerated	Antisense oligonucleot
Exocrine pancreatic insufficiency	Patients with exocrine pancreatic insufficiency due to cystic fibrosis	Fecal coefficient of fat absorption	Traditional	Reducing ar complexing
First aid antiseptic; Health care antiseptic; Consumer antiseptic	General public, consumers, and health care professionals	Bacterial count	Traditional and Monograph	Antimicrobia
Hepatitis A (Hep A) vaccine	Patients to be immunized against Hep A	Anti-Hep A virus antibody concentration	Traditional	Inactivated hepatitis A v vaccine Anti
Hepatitis B (Hep B) vaccine	Patients to be immunized against Hep B	Anti-Hep B virus antibody concentration	Traditional	Inactivated hepatitis B v vaccine Anti
Hepatitis B Virus (HBV)	Patients with HBV	Serum HBV DNA	Traditional	Antiviral
Hepatitis C Virus (HCV)	Patients with HCV with or without cirrhosis	Sustained viral response (HCV-RNA)	Traditional	Antiviral
Homozygous sitosterolemia (phytosterolemia)	Patients with homozygous sitosterolemia (phytosterolemia)	Plasma sitosterol and campesterol	Traditional	Dietary cholesterol absorption inhibitor
Human Immunodeficiency Virus-1 (HIV-1)	Patients with HIV-1	Undetectable plasma HIV-RNA	Traditional	Antiviral
Human Immunodeficiency Virus-1 (HIV-1)	Highly treatment experienced HIV-1 patients	Greater than 0.5 log reduction in plasma HIV RNA	Traditional	Antiviral
Hypercholesterolemia	Patients with heterozygous familial hypercholesterolemia	Serum LDL-C	Traditional	Lipid-lowerin
Hypercholesterolemia	Patients with homozygous familial hypercholesterolemia	Serum LDL-C	Traditional	Lipid-lowerin
Hyperphosphatemia	Patients chronic or end stage kidney disease on dialysis with hyperphosphatemia	Serum phosphate	Traditional	Phosphate b

Hypertension	Patients with hypertension	Blood pressure	Traditional	Angiotensin receptor blo Aldosterone antagonist
Hypertension	Patients with hypertension	Mean arterial pressure	Traditional	Vasodilator
Hypothyroidism	Patients with hypothyroidism	Thyroid-stimulating hormone (TSH)	Traditional	Thyroid horr analog
Influenza vaccine	Patients to be immunized against influenza	Hemagglutination inhibition antibody response	Accelerated	Inactivated influenza vir vaccine
Japanese encephalitis vaccine	Patients to be immunized against Japanese encephalitis	Neutralizing antibody response	Traditional	Inactivated Japanese encephalitis vaccine
Lipodystrophy	Patients with congenital or acquired generalized lipodystrophy	Serum hemoglobin A1C , fasting glucose and triglycerides	Traditional	Leptin analo
Melanoma	Patients with advanced melanoma	Durable Response Rate	Traditional	Mechanism unknown
Meningococcal A C Y W-135 vaccine	Patients to be immunized against meningococcal meningitis	Serum bactericidal antibody response	Traditional	Inactivated influenza vir vaccine
Meningococcal B vaccine	Patients to be immunized against meningococcal meningitis	Serum bactericidal antibody response	Traditional	Inactivated meningococ vaccine
Nephropathic cystinosis	Patients with nephropathic cystinosis	White blood cell cystine and serum creatinine#	Traditional	Cystine depl agent
Phenylketonuria	Patients with hyperphenylalaninemia due to tetrahydrobiopterin-responsive phenylketonuria	Plasma phenylalanine	Traditional	Phenylalanir hydroxylase activator
Polio vaccine	Patients to be immunized against polio	Neutralizing antibody response	Traditional	Inactivated poliovirus va

Precocious puberty	Patients with central precocious puberty	Serum luteinizing hormone	Traditional	Gonadotropi releasing hormone (G agonist
Pulmonary Tuberculosis (TB)	Patients with latent pulmonary TB	Time to sputum culture conversion to negative	Accelerated	Antimicrobia
Rabies immune globulin	Patients with suspected exposure to a rabid animal	Rabies neutralizing activity and antibody	Traditional	Inactivated r virus vaccine
Secondary hyperparathyroidism	Patients with secondary hyperparathyroidism associated with chronic kidney disease	Serum intact parathyroid hormone (iPTH)	Traditional	Vitamin D ar
Tetanus vaccine	Patients to be immunized against tetanus	Tetanus antitoxoid antibody	Traditional	Neutralizing antibody
Type 1 diabetes mellitus	Patients with type 1 diabetes mellitus	Serum hemoglobin A1C	Traditional	Glucose-low
Type 1 Gaucher disease	Patients with type 1 Gaucher disease	Spleen volume, liver volume, hemoglobin and platelet count#	Traditional	Hydrolytic lysozomal glucocerebr specific enzy
Type 2 diabetes mellitus	Patients with type 2 diabetes mellitus	Serum hemoglobin A1C	Traditional	Glucose-low
X-linked hypophosphatemia	Patients with X-linked hypophosphatemia	Serum phosphate	Traditional	Fibroblast gr factor 23 inh
Yellow fever vaccine	Patients at risk of exposure to yellow fever	Neutralizing antibody response	Traditional	Attenuated y fever virus vaccine

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