PILOT

CLINICAL OUTCOME ASSESSMENT COMPENDIUM

(COA Compendium)

Version 1

Last Updated:
Information Based on Drug Labeling Approved From 2003 to 2014: December 31, 2014; and
CDER's DDT COA Qualification Program: December 31, 2015

TABLE OF CONTENTS

COA COMPENDIUM OUTLINE DESCRIPTION

The *COA Compendium* is not a comprehensive list of clinical outcome assessments and is not intended to replace either existing disease-specific guidance or key interactions with FDA concerning drug development (e.g., during pre-IND meetings). Inclusion of a clinical outcome assessment in the *COA Compendium* is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.

Drug sponsors are strongly encouraged to seek advice from the relevant Office of New Drug (OND) review division early in drug development to discuss the selection and implementation of the clinical outcome assessment specific to their program, irrespective of whether the disease, condition, indication, claim, or clinical outcome assessment is included in the *COA Compendium*.

Some of the clinical outcome assessments listed in the *COA Compendium* may be protected by proprietary rights, and in some cases, a royalty and fee may be charged by the copyright owners for their authorized use. The inclusion of a clinical outcome assessment in the *COA Compendium* does not equate to an endorsement by FDA.

Please refer to the following COA Compendium website for limitations and use information: http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DevelopmentResources/default.htm

Clinical outcome assessments for the pilot version of the *COA Compendium* have been selected from two major sources:

- CDER's Drug Development Tool Clinical Outcome Assessment Qualification Program
- Drug Labeling Approved From 2003 to 2014 (New Molecular Entity (NME) labeling)

The pilot *COA Compendium* is organized by CDER's OND offices and review division assignments (e.g., the Division of Cardiovascular and Renal Products within the Office of Drug Evaluation I). The table alphabetically lists conditions or diseases based on each review division's therapeutic assignment. The table rows are color coded —specifically, the shaded rows describe information relating to a COA qualification project, whereas unshaded rows describe information relating to clinical outcome assessment from previous labeling.

The *COA Compendium* includes the following six columns:

COLUMNS	ELEMENTS	DESCRIPTION OF CONTENT
Column 1	**Disease/Condition**	Lists disease or condition and any relevant FDA disease-specific guidance.
Column 2	**Indication and/or Claim(s) Description**	Lists key elements of indication and/or claim (either labeled or qualified). For ongoing COA qualification projects, targeted labeling or promotional claim(s) may not be yet known and may be described as "to be determined." **Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a particular clinical*

		trial.
Column 3	**Outcome of Interest**	Describes an outcome of interest that was assessed (labeled) or could be assessed (in our qualification program) by clinical outcome assessment(s) displayed in Column 4.
Column 4	**COA (COA Type)**[1]	• Lists a labeled, qualified, or ongoing qualification project clinical outcome assessment name and/or description. • Includes the clinical outcome assessment type (i.e., a patient-reported outcome, observer-reported outcome, clinician-reported outcome, or performance outcome).
Column 5	**COA Context of Use**	Describes circumstances under which the outcomes of interest and the clinical outcome assessment have been used (i.e., labeled) or are targeted for use (i.e., they have been qualified or are part of an ongoing qualification).
Column 6	**COA Qualification Information**	Lists ongoing and completed clinical outcome assessment qualification project information, if applicable.

[1] *Note: Other outcomes such as biomarkers or survival may be listed if they were part of an overall composite assessment that included a COA.*

OFFICE OF ANTIMICROBIAL PRODUCTS (OAP)

ANTI-INFECTIVE PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[2] [3]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Acute bacterial exacerbation of chronic bronchitis in patients with chronic obstructive pulmonary disease (ABECB-COPD) Guidance for Industry: Acute Bacterial Exacerbations of Chronic Bronchitis in Patients With Chronic Obstructive Pulmonary Disease – Developing Antimicrobial Drugs for Treatment	Change in ABECB-COPD symptoms	Symptoms of ABECB-COPD	Exacerbation of Chronic Pulmonary Disease Tool (EXACT-PRO) (patient-reported outcome)	Outpatients with ABECB-COPD	Submitter: Evidera Guidance for Industry: Qualification of Exacerbations of Chronic Pulmonary Disease Tool for Measurement of Symptoms of Acute Bacterial Exacerbation of Chronic Bronchitis in Patients With Chronic Obstructive Pulmonary Disease (draft) Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Acute bacterial skin and skin structure infections (ABSSSI)[4] Guidance for Industry: Acute Bacterial Skin and Skin Structure Infections: Developing Drugs for Treatment	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Not applicable

[2] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[3] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.
[4] Outcome assessment related information was too complex to be amenable to a description in this COA Compendium's tabular format, please refer to the FDA issued guidance.

Disease/Condition	Indication and/or Claim(s) Description[2] [3]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Acute bacterial skin and skin structure infections (ABSSSI) Guidance for Industry: Acute Bacterial Skin and Skin Structure Infections: Developing Drugs for Treatment	To be determined	Symptoms of ABSSSI, and their impact on patient functioning	Acute Bacterial Skin and Skin Structure Infection (ABSSSI) PRO (patient-reported outcome)	Adult patients with a diagnosis of ABSSSI	Submitter: Foundation for the National Institutes of Health Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Acute otitis externa (AOE)	Treatment of AOE	Clinical cure based on absence of clinical signs (i.e., tenderness, erythema, and edema)	Composite of clinician-reported outcome for erythema and edema and patient-reported outcome for pain (4-point scale)	Pediatric and adult patients with acute otitis externa	Not applicable
		Clinical cure based on absence of clinical symptom (i.e., ear pain)			
		Microbiological eradication	Laboratory measure (biomarker)		
Clostridium difficile-associated diarrhea	Treatment of *Clostridium difficile*-associated diarrhea	Improvement in diarrhea or other symptoms (e.g., unformed stool count)	Patient diary capturing reduction in number of unformed stools (patient-reported outcome)	Adult patients with *Clostridium difficile*-associated diarrhea	Not applicable
		Sustained clinical response post initial treatment	Clinician-reported outcome and laboratory measure (biomarker)		
Community-acquired bacterial pneumonia (CABP)[5] Guidance for Industry: Community-Acquired Bacterial Pneumonia: Developing Drugs for Treatment (draft)	Refer to the draft Guidance for Industry	Refer to the draft Guidance for Industry	Refer to the Guidance for Industry	Refer to the draft Guidance for Industry	Not applicable

[5] Outcome assessment related information was too complex to be amenable to a description in this COA Compendium's tabular format, please refer to the FDA issued guidance.

Disease/Condition	Indication and/or Claim(s) Description[2] [3]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Community-acquired bacterial pneumonia (CABP) Guidance for Industry: Community-Acquired Bacterial Pneumonia - Developing Drugs for Treatment (draft)	To be determined	Symptoms of CABP, both respiratory and non-respiratory, and their impact on patients' functioning	Community Acquired Bacterial Pneumonia (CABP) PRO (patient-reported outcome)	Adult patients 18 years of age and older with a diagnosis of CABP For use in association with other disease specific endpoints to describe a treatment benefit for patients with CABP	Submitter: Foundation for the National Institutes of Health Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Complicated intra-abdominal infections[6] Guidance for Industry: Complicated Intra-Abdominal Infections – Developing Drugs for Treatment	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Not applicable
Complicated urinary tract infection[7] Guidance for Industry: Complicated Urinary Tract Infections – Developing Drugs for Treatment	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Refer to the Guidance for Industry	Not applicable
Hospital acquired bacterial pneumonia (HABP) Guidance for Industry: Hospital-Acquired Bacterial Pneumonia and Ventilator-Associated Bacterial Pneumonia – Developing Drugs for Treatment (draft)	To be determined	Symptoms of HABP and their effect on patients' functioning	Foundation for the National Institutes of Health (FNIH) Project - PRO measure for symptoms of HABP (patient-reported outcome)	Adult patients 18 years of age and older with a diagnosis of HABP	Submitter: FNIH and ICON Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[6] Outcome assessment related information was too complex to be amenable to a description in this COA Compendium's tabular format, please refer to the FDA issued guidance.

[7] Outcome assessment related information was too complex to be amenable to a description in this COA Compendium's tabular format, please refer to the FDA issued guidance.

Disease/Condition	Indication and/or Claim(s) Description[2] [3]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Impetigo[8]	Topical treatment of impetigo	Absence or improvement of treated lesions and no need for further antimicrobial treatment	Clinician-reported outcome	Adult and pediatric patients with impetigo	Not applicable
Leishmaniasis (cutaneous)	Treatment of cutaneous leishmaniasis	Epithelialization of lesions	Clinician-reported outcome	Adolescents and adult patients with cutaneous leishmaniasis	Not applicable
		No enlargement of lesions or no appearance of new lesions			
		Clearance of the parasites (assessment for parasites only if lesion persisted)			
Leishmaniasis (mucosal)	Treatment of mucosal leishmaniasis	Resolution of edema, erythema, infiltration and erosion from the involved mucosal sites	Clinician-reported outcome	Adolescents and adult patients with mucosal leishmaniasis	Not applicable
Leishmaniasis (visceral)	Treatment of visceral leishmaniasis	Clearance of the parasites	Clinician-reported outcome and laboratory measure (biomarker)	Adolescents and adult patients with visceral leishmaniasis	Not applicable
		Resolution of the signs and symptoms of the disease			
Travelers' diarrhea	Treatment of travelers' diarrhea caused by noninvasive strains of *Escherichia coli*	Clinical response based on time to last unformed stool	Patient diary (patient-reported outcome) and laboratory measure (biomarker)	Patients with travelers' diarrhea caused by noninvasive strains of *Escherichia coli*	Not applicable
		Resolution of symptoms			
		Microbiological eradication			

[8]Sponsors interested in the development of drugs for treatment of impetigo or minor cutaneous abscess should discuss their development plans with the FDA. In general, such trials should be designed for a finding of superiority (see the transcripts of the discussion at the November 18, 2008, Anti-Infective Drugs Advisory Committee (AIDAC) meeting).

ANTIVIRAL PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[9] [10]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Influenza Guidance for Industry: Influenza – Developing Drugs for Treatment and/or Prophylaxis	Treatment of acute uncomplicated influenza	Symptoms improvement (e.g., cough, sore throat, nasal congestion, headache, feverishness, myalgia and fatigue)	Patient diary (patient-reported outcome)	Adult patients with acute uncomplicated influenza	Not applicable
Influenza Guidance for Industry: Influenza – Developing Drugs for Treatment and/or Prophylaxis	To be determined	Presence and severity of patient-reported influenza symptoms	Flu-PRO (patient-reported outcome)	Pediatric and adult patients with documented influenza	Submitter: National Institute of Allergy and Infectious Diseases, Leidos Biomedical Research, Department of Defense, and Evidera Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[9] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[10] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

TRANSPLANT AND OPHTHALMOLOGY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[11] [12]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Allergic conjunctivitis (itching associated with allergic conjunctivitis)	Prevention of itching associated with allergic conjunctivitis	Ocular itching severity	5-point itching severity numerical rating scale with half unit increments (patient-reported outcome)	Pediatric and adult patients with itching associated with allergic conjunctivitis	Not applicable
Bacterial conjunctivitis	Treatment of bacterial conjunctivitis	Clinical resolution based on absence of all three clinical signs (e.g., ocular discharge, bulbar conjunctival injection, and palpebral conjunctival injection)	Clinician-reported outcome	Use in pediatric and adult patients with bacterial conjunctivitis along with other key efficacy measures (e.g., microbiology)	Not applicable
		Microbiological eradication	Laboratory measure (biomarker)		
Kidney transplantation (prophylaxis of organ rejection)	Prophylaxis of organ rejection in adult patients receiving a kidney transplant	Incidence of biopsy proven acute rejection	Composite of clinician-reported outcomes, survival, biomarker, and patient loss to follow up	Patients with kidney transplant	Not applicable
		Graft loss			
		Death			
		Patient loss to follow up			
		Glomerular filtration	Laboratory measure (biomarker)		
Neovascular (wet) age-related macular degeneration	Treatment of age-related macular degeneration	Best corrected visual acuity	Visual acuity (performance outcome)	Adult patients with age-related macular degeneration	Not applicable

[11] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[12] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[11] [12]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Ocular inflammation and pain associated with ocular surgery	Treatment of ocular inflammation and pain associated with ocular surgery	Absence of post-surgical ocular inflammation	Slit lamp evaluation of the affected eye(s) (clinician-reported outcome) with inflammation measured by anterior chamber cell/flare grade score	Adult patients undergoing ocular surgery	Not applicable
		Absence of post-surgical ocular pain/ discomfort	Visual analog scale and/or 6-point numeric pain rating scale (patient-reported outcome)		
Ophthalmic surgery aid	Aid in ophthalmic surgery by staining the anterior capsule of the lens	Anterior lens capsule staining	Biomicroscopy (clinician-reported outcome)	Patients undergoing ophthalmic surgical procedures	Not applicable
Vitreomacular adhesion	Treatment of symptomatic vitreomacular adhesion	Nonsurgical vitreomacular adhesion resolution	Biomicroscopy (clinician-reported outcome) using 4-point scale	Use in adult patients with symptomatic vitreomacular adhesion	Not applicable
		Best corrected visual acuity	Visual acuity (performance outcome)		

OFFICE OF DRUG EVALUATION I (ODE I)

CARDIOVASCULAR AND RENAL PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[13] [14]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Acute coronary syndrome (ACS)	To reduce the rate of thrombotic cardiovascular events in patients with ACS	Incidence of cardiovascular death, non-fatal myocardial infarction, non-fatal stroke	Composite of clinician-reported outcomes	Adult patients with ACS	Not applicable
Atrial fibrillation (AF) / atrial flutter (AFL)	To reduce the risk of symptomatic recurrence of AF/AFL in patients in sinus rhythm with a history of paroxysmal or persistent AF/AFL	Hospitalization due to cardiovascular cause or death from any cause	Clinician-reported outcome or survival	Adult patients in sinus rhythm with a history of paroxysmal or persistent atrial fibrillation / atrial flutter	Not applicable
Chronic stable angina	Treatment of chronic stable angina	Exercise duration	Modified Bruce Treadmill Exercise test (performance outcome)	Adult patients with chronic angina	Not applicable
		Angina attack frequency	Patient diary (patient-reported outcome) – measures frequency		
		Nitroglycerine use	Patient diary (patient-reported outcome)		
Chronic thromboembolic pulmonary hypertension (CTEPH)	Treatment of persistent/recurrent CTEPH after surgical treatment or inoperable CTEPH to improve exercise capacity and WHO functional class	Exercise capacity	6-Minute Walking Distance (performance outcome)	Adult patients with CTEPH	Not applicable
		World Health Organization functional class / lack of deterioration (deterioration by itself is not adequate)	World Health Organization functional assessment (clinician-reported outcome)		
Heart failure syndrome	To be determined	Symptoms of heart failure syndrome and their impact on physical limitations	Kansas City Cardio-Myopathy Questionnaire (patient-reported outcome)	Adult patients with heart failure syndrome	Mid America Heart Institute Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[13] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[14] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[13] [14]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Neurogenic orthostatic hypotension	Treatment of orthostatic dizziness, lightheadedness, or the "feeling that you are about to blackout"	Symptom severity (e.g., dizziness, lightheadedness, feeling faint, and "feeling like you might blackout")	Orthostatic Hypotension Questionnaire Item #1 (patient-reported outcome)	Adult patients with symptomatic neurogenic orthostatic hypotension and	Not applicable
Non-valvular atrial fibrillation	To reduce the risk of stroke and systemic embolism in patients with non-valvular atrial fibrillation	Incidence of stroke and systemic embolism	Composite of clinician-reported outcomes	Adult patients with non-valvular atrial fibrillation	Not applicable
Pulmonary arterial hypertension	Treatment of pulmonary arterial hypertension	Exercise capacity	6-Minute Walking Distance (performance outcome)	Adult patients with pulmonary arterial hypertension	Not applicable
		Incidence of death or clinical deterioration	Survival and/or clinician-reported outcome		
Patients with a history of atherosclerosis	Reduction of thrombotic cardiovascular events in patients with a history of myocardial infarction or peripheral arterial disease	Incidence of cardiovascular death, myocardial infarction, and stroke	Composite of clinician-reported outcomes	Adult patients with a history of atherosclerosis involving the coronary, cerebral, or peripheral vascular systems	Not applicable
		Avoidance of hospitalization for unstable angina			
		Avoidance of coronary revascularization			
Varicose veins	Treatment of uncomplicated spider veins and uncomplicated reticular veins in the lower extremity	Improvement of varicose veins (symptoms and appearance)	Digital photographs of the treatment area and 5-point verbal rating scale (clinician-reported outcome)	Adult patients with spider or reticular varicose veins	Not applicable
		Patient satisfaction with treatment	5-point verbal rating scale (patient-reported outcome)		
Varicose veins	To be determined	Varicose vein symptoms	Varicose Vein Symptom Questionnaire (VVSymQ) (patient-reported outcome)	Adult patients with superficial venous incompetence	Submitter: BTG International Inc. Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

NEUROLOGY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[15] [16]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Alzheimer's disease (AD)	Treatment of moderate to severe dementia of the AD	Day-to-day function	Modified Alzheimer's Disease Cooperative Study-Activities of Daily Living (clinician-reported outcome)	Adult patients with moderate to severe AD	Not applicable
		Cognitive function	Severe Impairment Battery (performance outcome)		
		Global impression	Clinical Global Impression of Change (clinician-reported outcome)		
Alzheimer's disease: Mild cognitive impairment due to Alzheimer's disease (MCI due to AD) Guidance for Industry: Alzheimer's Disease - Developing Drugs for the Treatment of Early Stage Disease (draft)	To be determined	Day-to-day functioning (instrumental activities of daily living)	Currently unnamed (performance outcome tool to assess instrumental activities of daily living (IADLs))	Adults (≥45 years) with mild cognitive impairment due to Alzheimer's disease (MCI due to AD)	Submitter: Critical Path Institute: PRO Consortium's Cognition Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Blepharospasm	Treatment of blepharospasm	Signs and symptoms	Jankovic Rating Scale – severity domain (clinician-reported outcome)	Adult patients with blepharospasm	Not applicable
Cervical dystonia	Treatment of cervical dystonia	Severity of dystonia	Toronto Western Spasmodic Torticollis Rating Scale (composite of– clinician-reported outcome and patient-reported outcome)	Adult patients with cervical dystonia	Not applicable
		Disability			
		Pain			

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[16] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[15] [16]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Duchenne muscular dystrophy (DMD) Guidance for Industry: Duchenne Muscular Dystrophy and Related Dystrophinopathies – Developing Drugs for Treatment (draft)	To be determined	Functional reaching volume intended to encompass upper extremity and trunk movement	Abilities Captured through Interactive Video Evaluation (ACTIVE)-seated (performance outcome)	Children (4 years of age and older) and adults with dystrophinopathy both DMD and Becker muscular dystrophy (BMD)	Submitter: The Research Institute at Nationwide Children's Hospital Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Huntington's disease (HD)	Treatment of chorea associated with HD	Chorea	Total chorea score of the Unified Huntington's Disease Rating Scale (UHDRS) (clinician-reported outcome)	Adult patients with chorea associated with HD	Not applicable
		Global impression	Clinical Global Impression (clinician-reported outcome)		
Multiple sclerosis (MS)	Improve walking in patients with MS	Walking speed	Timed 25-Foot Walk (performance outcome)	Adult patients with MS	Not applicable
		Ambulatory disability	12-Item Multiple Sclerosis Walking Scale (patient-reported outcome)		
Multiple sclerosis (MS)	Treatment of patients with relapsing forms of MS	Relapse frequency	Clinician-reported outcome	Adult patients with relapsing forms of MS	Not applicable
		Physical disability	Expanded Disability Status Scale (clinician-reported outcome)		
Multiple sclerosis (MS)	To be determined	"MS disability", or simply "disability", characterized as neurological or neuropsychological deficits that result in limitation in activities, participation, or roles caused by MS that are understood to be important	New Clinical Outcome Assessment Instrument for Use in Clinical Trials of Medical Products to Treat Multiple Sclerosis (MS) (performance outcome)	Adults living with relapsing-remitting or progressive forms of MS	Submitter: Critical Path Institute Multiple Sclerosis Outcome Assessments Consortium (MSOAC) Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Non-24-hour sleep-wake disorder	Treatment of non-24 hour sleep wake disorder	Nighttime sleep time	Patient diary (patient-reported outcome)	Adult patients with non-24 hour sleep wake disorder	Not applicable
		Daytime nap time			

Disease/Condition	Indication and/or Claim(s) Description[15] [16]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Parkinson's disease (PD) (Early stage)	Treatment of PD (early stage)	Motor findings	Unified Parkinson's Disease Rating Scale (UPDRS) – Part III (clinician-reported outcome)	Adult patients with PD (early stage)	Not applicable
		Activities of daily living	UPDRS – Part II (clinician-reported outcome)		
Parkinson's disease (PD) (Advanced stage)	Treatment of PD (advanced stage)	"Off" time	Patient diary (patient-reported outcome)	Adult patients with PD (advanced stage)	Not applicable
		Motor findings	Unified Parkinson's Disease Rating Scale (UPDRS) – Part III (clinician-reported outcome)		
		Activities of daily living	UPDRS – Part II (clinician-reported outcome)		
Restless legs syndrome (RLS)	Treatment of moderate to severe RLS	Sensory and motor symptom severity and impacts on sleep, daytime tiredness or sleepiness, activities of daily living, and mood associated with RLS	International Restless Legs Syndrome Rating Scale (patient-reported outcome)	Adult patients with moderate to severe primary RLS	Not applicable
		Global impression – RLS symptoms change	Clinical global impression of improvement (clinician-reported outcome)		
Seizure disorder: Infantile spasms	Treatment of infantile spasms	Electroencephalogram (EEG)-cessation of hypsarrhythmia	Video/electroencephalogram (EEG) (clinician-reported outcome)	Pediatric (1 month – 2 years) patients with infantile spasms	Not applicable
		Complete cessation of seizures	Patient diary (observer-reported outcome)		
Seizure disorder: Lennox-Gastaut Syndrome (LGS)	Treatment of LGS	Seizure frequency	Patient diary (observer-reported outcome and/or patient-reported outcome as appropriate)	Pediatric (1 year and up) and adult patients with LGS	Not applicable

Disease/Condition	Indication and/or Claim(s) Description[15] [16]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Seizure disorder: Partial-Onset Seizures (POS)	Treatment of POS	Seizure frequency	Patient diary (observer-reported outcome and/or patient-reported outcome as appropriate)	Pediatric (2 years and up) and adult patients with POS	Not applicable
			Video/electroencephalogram (EEG) frequency measure (clinician-reported outcome)	Pediatric (1 month to 4 years) patients with POS	
Seizure disorder: Refractory Complex Partial Seizures	Treatment of refractory complex partial seizures	Seizure frequency	Patient diary (observer-reported-outcome and/or patient-reported outcome as appropriate)	Pediatric (2 years and up) and adult patients with refractory complex partial seizures	Not applicable

PSYCHIATRY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[17] [18]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Attention-deficit/hyperactive disorder (ADHD)	Treatment of ADHD	Signs and symptoms of ADHD	• ADHD Rating Scale (clinician-reported outcome) • Connor's Parent Rating Scale (observer-reported outcome) • Permanent Product Measure of Performance (performance outcome)	Pediatric and adult patients with ADHD	Not applicable
		Behavior (patient deportment)	Swanson, Kotkin, Angler, M-Flynn, and Pelham Deportment Scores (clinician-reported outcome)		
		Global impression	Clinical Global Impression – Improvement (clinician-reported outcome)		
		Global impression	Clinical Global Impression – severity (clinician-reported outcome)		

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[18] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[17] [18]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Bipolar disorder	Treatment of acute manic or mixed episodes associated with bipolar I disorder	Manic symptoms (e.g., irritability, speech, thought content, and disruptive/aggressive behavior)	Young-Mania Rating Scale (clinician-reported outcome)	Adult patients with bipolar I disorder with acute or mixed episodes with or without psychotic features	Not applicable
		Global impression	Clinical Global Impression – Bipolar Severity of Illness score (clinician-reported outcome)		
Insomnia	Treatment of insomnia characterized by difficulty with sleep onset and/or maintenance	Sleep latency (sleep onset) and/or wake time after sleep onset (sleep maintenance)	Patient diary (patient-reported outcome) and polysomnography (biomarker)	Adult patients with insomnia characterized by difficulty with sleep onset and/or maintenance	Not applicable
Major depressive disorder (MDD)	Treatment of MDD	Symptoms of MDD	• Montgomery-Asberg Depression Rating Scale (clinician-reported outcome) or • Hamilton Depression Rating Scale 24 items or 17 items (clinician-reported outcome)	Adult patients with MDD	Not applicable
		Global impression	Clinical Global Impression scale (clinician-reported outcome)		
		Relapse rate	Clinical Global Impression –severity (clinician-reported outcome)		
Major depressive disorder (MDD)	To be determined	Symptoms of MDD	Symptoms of Major Depressive Disorder Scale (SMDDS) (patient-reported outcome)	Adult patients with MDD	Submitter: Critical Path Institute: PRO Consortium's Depression Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

Disease/Condition	Indication and/or Claim(s) Description[17] [18]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Schizophrenia	Treatment of schizophrenia	Signs and symptoms – positive	Brief Psychiatric Rating Scale (clinician-reported outcome)	Adult patients with schizophrenia	Not applicable
		Signs and symptoms – positive and negative	Positive And Negative Syndrome Scale (clinician-reported outcome)		
		Global impression	Clinical Global Impression severity scale (clinician-reported outcome)		
		Personal and social functioning	Personal and Social Performance scale (clinician-reported outcome)		
		Relapse rate	Positive and Negative Syndrome Scale (clinician-reported outcome)		
			Clinical Global Impression – Severity of Illness score (clinician-reported outcome)		

OFFICE OF DRUG EVALUATION II (ODE II)

ANESTHESIA, ANALGESIA, AND ADDICTION PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[19] [20]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Alcoholism Guidance for Industry: Alcoholism – Developing Drugs for Treatment (draft)	Treatment of alcohol dependence	Attaining and maintaining abstinence or low risk drinking (e.g., frequency of alcohol consumption, quantity of alcohol consumed, and laboratory measure)	Patient-reported outcome and laboratory confirmation (biomarker)	Adult patients who are alcohol-dependent	Not applicable
Chronic musculoskeletal pain *Refer to the following draft guidance for industry for specific type of chronic musculoskeletal pain Guidance for Industry: Analgesic Indications: Developing Drug and Biological Products (draft)	Treatment of chronic musculoskeletal pain	Pain intensity	Numerical pain rating scale or visual analog scale (patient-reported outcome)	Patients with chronic musculoskeletal pain	Not applicable
Pain (acute) Guidance for Industry: Analgesic Indications – Development Drug and Biological Products (draft)	Treatment of acute pain	Pain intensity	Numerical pain rating scale or visual analog scale (patient-reported outcome)	Patients with acute pain	Not applicable

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[20] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[19] [20]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Pain (chronic) *Refer to the following draft guidance for industry for specific type of chronic pain Guidance for Industry: Analgesic Indications – Development Drug and Biological Products (draft)	Treatment of chronic pain	Pain intensity	Numerical pain rating scale or visual analog scale (patient-reported outcome)	Patients with chronic pain	Not applicable
Pain (neuropathic) *Refer to the following draft guidance for industry for specific type of neuropathic pain Guidance for Industry: Analgesic Indications – Development Drug and Biological Products (draft)	Treatment of neuropathic pain	Pain intensity	Numerical pain rating scale or visual analog scale (patient-reported outcome)	Patients with neuropathic pain	Not applicable
Pain (acute or chronic) Guidance for Industry: Analgesic Indications – Development Drug and Biological Products (draft)	To be determined	Pain intensity	QUALIfied Therapeutic Evaluations of Pain (QUALITE-Pain) (patient-reported outcome)	Non-cognitively impaired adolescents and adults (ages ≥ 12 years) with acute or chronic pain	Submitter: QUALITE-Pain (Univ. of Rochester; Univ. of Washington) Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Smoking cessation	Aid to smoking cessation treatment	Abstinence	Patient-reported abstinence and laboratory measure (biomarker)	Current smokers	Not applicable

METABOLISM AND ENDOCRINOLOGY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[21] [22]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Human immunodeficiency virus (HIV)-related lipodystrophy	Improvement in belly appearance distress	Body image	Belly Appearance Distress Score (patient-reported outcome)	Adult patients with HIV-related lipodystrophy	Not applicable
Muscle wasting disorder (lower extremity functional decline in patients with hip fracture)	To be determined	Lower-extremity functional decline	Usual Gait Speed (UGS) and the Short Physical Performance Battery (SPPB) (performance outcome)	Persons age 65 years and older who have diminished muscle mass and strength and decreased function that is a result of a hip fracture	Submitter: Aging in Motion Coalition of the Alliance for Aging Research Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Sarcopenia	To be determined	Physical functioning	Patient Reported Outcome Measurement System (PROMIS) – Physical Function item bank (patient-reported outcome)	Adult patients with sarcopenia	Submitter: PROMIS Network Center Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Weight-related quality of life	To be determined	Improvements in specific aspects of health related quality of life following weight loss	Impact of Weight on Quality of Life-Lite (IWQOL-Lite) Clinical Trials Version (patient-reported outcome)	Adult patients who are overweight and obese	Submitter: Quality of Life Consulting and Duke University Medical Center Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[21] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[22] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

PULMONARY, ALLERGY, AND RHEUMATOLOGY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Allergic rhinitis Guidance for Industry: Allergic Rhinitis – Clinical Development Programs for Drug Products (draft)	Treatment of nasal symptoms associated with seasonal and perennial allergic rhinitis	Nasal symptoms severity (e.g., runny nose, nasal itching, sneezing, and nasal congestion)	4-point categorical nasal symptom severity scale (patient-reported outcome)	Pediatric and adult patients with allergic rhinitis	Not applicable
Ankylosing spondylitis	Treatment of ankylosing spondylitis	Signs and symptoms of ankylosing spondylitis (e.g., pain, inflammation)	Assessment in Ankylosing Spondylitis (ASAS 20) as a composite measure consisting of patient-reported outcomes (visual analog pain scale, Bath Ankylosing Spondylitis Functional Index, Bath Ankylosing Spondylitis Disease Activity Index, and patient global assessment)	Adult patients with ankylosing spondylitis	Not applicable
Asthma	Treatment of moderate to severe persistent asthma	Frequency and severity of asthma exacerbation	Patient diary (patient-reported outcome)	Pediatric and adult patients with asthma	Not applicable
		Lung function	Forced expiratory volume in one second using spirometry (FEV1)[25]		
		Asthma symptoms severity	Asthma symptoms score (patient-reported outcome)		

[23] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[24] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.
[25] FEV1/FVC-based outcome assessments classification may evolve as FDA refines clinical outcome assessment and biomarker definitions in the future.

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Asthma	To be determined	Asthma symptoms	Asthma Daily Symptom Diary (ADSD) (patient-reported outcome)	Adolescents (12 to 17 years) and adults (≥18 years) with mild to severe persistent asthma	Submitter: Critical Path Institute: PRO Consortium's Asthma Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Chronic obstructive pulmonary disease (COPD) Guidance for Industry: Chronic Obstructive Pulmonary Disease – Developing Drugs for Treatment (draft)	Treatment of COPD airflow obstruction	Lung function	Forced expiratory volume in one second using spirometry (FEV1)[26]	Adult patients with COPD	Not applicable
		Symptoms frequency and severity, physical activity limitations, and impact on daily life (health-related quality of life)	St. George's Respiratory Questionnaire (patient-reported outcome)		
Chronic obstructive pulmonary disease (COPD) Guidance for Industry: Chronic Obstructive Pulmonary Disease – Developing Drugs for Treatment (draft)	Reducing the risk of COPD exacerbations	Incidence of moderate/severe exacerbations	Patient diary (patient-reported outcome)	Adult patients with COPD	Not applicable
		Lung function	Forced expiratory volume in one second using spirometry (FEV1)[27]		

[26] FEV1/FVC-based outcome assessments classification may evolve as FDA refines clinical outcome assessment and biomarker definitions in the future.
[27] FEV1/FVC-based outcome assessments classification may evolve as FDA refines clinical outcome assessment and biomarker definitions in the future.

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Chronic obstructive pulmonary disease (COPD) Guidance for Industry: Chronic Obstructive Pulmonary Disease – Developing Drugs for Treatment (draft)	To be determined	Respiratory symptom severity	Exacerbations of Chronic Pulmonary Disease Tool - Respiratory Symptoms (EXACT-RS) (patient-reported outcome)	Patients with COPD	Submitter: Evidera Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Cryopyrin-associated periodic syndromes (CAPS)	Treatment of CAPS	Signs and symptoms severity (e.g., rash, fatigue, joint pain, feeling of fever/chills, and eye redness/pain)	Patient diary based on 11-point numeric rating severity scale for each symptom/sign (patient-reported outcome)	Pediatric and adult patients with CAPS	Not applicable
Cryopyrin-associated periodic syndromes (CAPS)	Treatment of CAPS	Physician's assessment of disease activity Physician's assessment of skin disease Inflammation	Complete clinical response as a composite measure consisting of clinician-reported outcomes and laboratory measures (i.e., C-Reactive Protein and Serum Amyloid A) (biomarkers)	Pediatric and adult patients with CAPS	Not applicable

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Cystic fibrosis	Treatment of cystic fibrosis	Lung function	Forced expiratory volume in one second using spirometry (FEV1)[28]	Pediatric and adult patients with cystic fibrosis	Not applicable
		Respiratory symptoms severity	Cystic Fibrosis Questionnaire-Revised respiratory domain (patient-reported outcome)		
		Pulmonary exacerbation	Clinician-reported outcome based on treatment change		
		Body weight and sweat chloride	Laboratory and weight scale (biomarkers)		
Cystic fibrosis	To be determined	Severity of symptoms of cystic fibrosis	Cystic Fibrosis Respiratory Symptom Diary – Chronic Respiratory Infection Symptom Score (CFRSD-CRISS) (patient-reported outcome)	Adults and adolescents (≥12 years) with a chronic respiratory infection in stable patients and patients with an acute exacerbation	Submitter: Evidera and Cystic Fibrosis Foundation Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Hereditary angioedema (HAE)	Treatment of acute attacks of HAE	Signs and symptom severity (e.g., skin swelling, skin pain, abdominal pain)	Sign and symptom severity measured using either: • Visual analog scale (patient-reported outcome) or • Categorical scale (Mean Symptom Complex Severity score) (patient-reported outcome)	Pediatric and adult patients with HAE	Not applicable
		Improvement of each anatomic site of attack	Categorical scale evaluating treatment improvement (Treatment Outcome Score) (patient-reported outcome)		

[28] FEV1/FVC-based outcome assessments classification may be evolve as FDA refines clinical outcome assessment and biomarker definitions in the future.

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Idiopathic pulmonary fibrosis	Treatment of idiopathic pulmonary fibrosis (IPF)	Lung function	Forced vital capacity assessment using spirometry (FVC)[29]	Adult patients with idiopathic pulmonary fibrosis	Not applicable
		IPF exacerbation (defined as worsening or development of dyspnea and imaging abnormalities)	Clinician-reported outcome and imaging (biomarker)		
Idiopathic pulmonary fibrosis	To be determined	Symptoms severity	A Tool to Assess Quality of Life in Idiopathic Pulmonary Fibrosis (ATAQ-IPF) (patient-reported outcome)	Adult patients with idiopathic pulmonary fibrosis	Submitter: National Jewish Health Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Psoriatic arthritis	Treatment of active psoriatic arthritis	Improvement in number of tender and swollen joints	American College of Rheumatology (ACR) core set of outcome measures (composite measures of clinician-reported outcomes, patient-reported outcomes, and biomarker)	Adult patients with active psoriatic arthritis	Not applicable
		Pain intensity			
		Patient's global assessment of disease activity			
		Physician's global assessment of disease activity			
		Disability/physical functioning			
		Laboratory measure (e.g., sedimentation rate)			
Respiratory distress syndrome (RDS)	Prevention of RDS in premature infants at high risk for RDS	Incidence of RDS	Clinician-reported outcome	Premature infants at high risk for RDS	Not applicable
		RDS-related mortality			

[29] FEV1/FVC-based outcome assessments classification may evolve as FDA refines clinical outcome assessment and biomarker definitions in the future.

Disease/Condition	Indication and/or Claim(s) Description[23] [24]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Rheumatoid arthritis (RA) Guidance for Industry: Rheumatoid Arthritis – Developing Drug Products for Treatment (draft)	Treatment of RA	Improvement in number of tender and swollen joints	American College of Rheumatology (ACR) core set of outcome measures (composite measures of clinician-reported outcomes, patient-reported outcomes, and biomarker)	Adult patients with RA	Not applicable
		Pain intensity			
		Patient's global assessment of disease activity			
		Physician's global assessment of disease activity			
		Disability/physical functioning			
		Laboratory measure (e.g., sedimentation rate)			
		Structural damage	Imaging (biomarker)		
		General health status domains	Short Form (SF-36) (patient-reported outcome)		
Rheumatoid arthritis (RA) Guidance for Industry: Rheumatoid Arthritis – Developing Drug Products for Treatment (draft)	To be determined	Fatigue	Currently unnamed (PRO measure to assess fatigue) (patient-reported outcome)	Adults (≥18 years) with RA with mild to severe disease activity	Submitter: Critical Path Institute Rheumatoid Arthritis Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Systemic lupus erythematosus Guidance for Industry: Systemic Lupus Erythematosus – Developing Medical Products for Treatment	Treatment of systemic lupus erythematosus	Signs and symptoms and disease activity severity	Composite of Systemic Lupus Erythematosus Disease Activity Index, British Isles Lupus Activity Group, and Physician's Global Assessment of no worsening (clinician-reported outcomes)	Patients with systemic lupus erythematosus	Not applicable
		Global impression - assessing no worsening			

OFFICE OF DRUG EVALUATION III (ODE III)

DERMATOLOGY AND DENTAL PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[30] [31]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Actinic keratosis (topical therapy)	Treatment of actinic keratosis (face, scalp, trunk, and extremities)	Clearance of actinic keratosis lesions	Clinician-reported outcome	Adult patients with actinic keratosis	Not applicable
External genital and perianal warts (topical therapy)	Treatment of external genital and perianal warts	Clearance of external genital and perianal warts	Clinician-reported outcome	Adult patients with external genital and perianal warts	Not applicable
Head lice infestations (topical therapy)	Treatment of head lice infestations	Absence of live lice	Clinician-reported outcome	Pediatric and adult patients with head lice infestations	Not applicable
Interdigital tinea pedis, tinea cruris, tinea corporis (topical therapy)	Treatment of interdigital tinea pedis, tinea cruris, and/or tinea corporis	Clearance of signs and symptoms (e.g., erythema, scaling, and pruritus)	Clinician reported outcome Note: pruritus symptoms are assessed based on patient-reported outcome	Pediatric and/or adult patients with interdigital tinea pedis, tinea cruris, and/or tinea corporis	Not applicable
		Fungal culture and potassium hydroxide (KOH) tests	Laboratory measure (biomarkers)		
Onychomycosis (topical therapy)	Treatment of onychomycosis	Clinical evidence of the disease (absence of signs/symptoms)	Composite assessment of clinician-reported outcome and laboratory measures (biomarkers)	Adult patients with onychomycosis	Not applicable
		Fungal culture and potassium hydroxide (KOH) tests			

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[31] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[30] [31]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Plaque psoriasis (systemic therapy)	Treatment of moderate to severe plaque psoriasis	Psoriasis area and severity	Psoriasis Area and Severity Index (PASI) (clinician-reported outcome)	Adult patients with moderate to severe plaque psoriasis	Not applicable
		Global assessment of the overall signs of the disease (e.g., plaque thickness/induration, erythema, and scaling)	Categorical Static Physician's Global Assessment scale (clinician-reported outcome)		

GASTROENTEROLOGY AND INBORN ERRORS PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[32] [33]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Chronic idiopathic constipation	Treatment of chronic idiopathic constipation	Complete spontaneous bowel movements frequency	Complete spontaneous bowel movement patient diary (patient-reported outcome)	Adult patients with chronic idiopathic constipation	Not applicable
		Signs and symptoms related to constipation (e.g., abdominal pain, stool consistency)	Numeric rating scale assessing signs and symptoms (patient-reported outcomes) Note: Bristol Stool Form Scale for assessment of stool consistency (patient-reported outcome)		
Colonoscopy	Cleaning of the colon as a preparation for colonoscopy procedure	Successful excellent bowel prep (visualization of mucosa and minimal need for additional washing)	Clinician-reported outcome	Adult patients scheduled for elective colonoscopy	Not applicable
Crohn's disease (CD)	To be determined	Signs and symptoms of CD	Crohn's Disease Patient-Reported Outcomes Signs and Symptoms (CD-PRO/SS) (patient-reported outcome)	Adult patients with moderate to severe CD in the outpatient setting	Submitter: Evidera Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Crohn's disease (CD) (pediatric)	To be determined	Disease activity in pediatric patients with CD	TUMMY-CD (patient-reported outcome)	Pediatric patients (<18 years of age) with CD	Submitter: IWK Health Centre (Canada) Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

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[33] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[32] [33]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Eosinophilic esophagitis (EoE)	To be determined	Esophageal symptoms	Pediatric Eosinophilic Esophagitis Score (PEES v2.0) (patient-reported outcome)	Children and adolescent patients (ages 8 to 18 years) with EoE	Submitter: Marc Rothenberg Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Functional dyspepsia (FD)	To be determined	Symptoms of functional dyspepsia	Functional Dyspepsia Symptom Diary (FDSD) (patient-reported outcome)	Adults (≥18 years) that have met Rome III diagnostic criteria for FD	Submitter: Critical Path Institute PRO Consortium's Functional Dyspepsia Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Gastroparesis Guidance for Industry: Gastroparesis: Clinical Evaluation of Drugs for Treatment (draft)	To be determined	Symptoms of gastroparesis	Gastroparesis Cardinal Symptom Index Daily Diary (ANMS GCSI) (patient-reported outcome)	Outpatients who are 18 years or older who have been diagnosed with idiopathic or diabetic gastroparesis	Submitter: American Neurogastroenterology and Motility Society Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Gaucher (type 2 and 3)	To be determined	Severity of neurological signs and symptoms (neurological disease burden)	Gaucher disease COA (COA type to be determined)	Patients aged 1 and older with neuronopathic Gaucher disease (nGD)	Submitter: Baylor Research Institute Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

Disease/Condition	Indication and/or Claim(s) Description[32] [33]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Irritable bowel syndrome – constipation (IBS-C) Guidance for Industry: Irritable Bowel Syndrome – Clinical Evaluation of Drugs for Treatment	Treatment of IBS-C	Complete spontaneous bowel movement frequency	Patient diary(patient-reported outcome)	Adult patients with IBS-C	Not applicable
		Abdominal pain intensity	11-point abdominal pain numeric rating scale (patient-reported outcome)		
Irritable bowel syndrome (IBS-C, IBS-D, and IBS-M) Guidance for Industry: Irritable Bowel Syndrome – Clinical Evaluation of Drugs for Treatment	To be determined	Signs and Symptoms of IBS (IBS-C, IBS-D, and IBS-M)	Currently unnamed (three PRO measures to assess the signs and symptoms of the three main subtypes of IBS: IBS-C, IBS-D, and IBS-M) (patient-reported outcome)	Adults (≥18 years) with IBS-C, IBS-D, or IBS-M	Submitter: Critical Path Institute PRO Consortium's IBS Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Mucopolysaccharidosis I (MPS I) (Hurler and Hurler-Scheie forms of MPS I)	Improvement in walking capacity	Walking capacity	6-Minute Walk Test (performance outcome)	Pediatric and/or adult patients with MPS I	Not applicable
Mucopolysaccharidosis II (MPS II) (Hunter syndrome)	Improvement in walking capacity	Walking capacity	6-Minute Walk Test (performance outcome)	Pediatric and/or adult patients with MPS II	Not applicable
Mucopolysaccharidosis VI (MPS VI) (Maroteaux-Lamy syndrome)	Improvement in walking and stair-climbing capacity	Walking capacity	12-Minute Walk Test (performance outcome)	Pediatric and/or adult patients with MPS VI	Not applicable
		Stair-climbing capacity	3-Minute Stair Climb Test (performance outcome)		
Nausea and vomiting associated with chemotherapy	Prevention of chemotherapy-induced nausea and vomiting (acute and delayed)	Absence of vomiting or retching AND no use of rescue medication	Patient diary (patient-reported outcomes)	Adult patients at risk for nausea and vomiting associated with emetogenic chemotherapy	Not applicable
Non-infectious diarrhea	Symptomatic relief of non-infectious diarrhea in patients with HIV/AIDS on antiretroviral therapy	Consistency of the bowel movement	• Bristol Stool Form Scale (patient-reported outcome) • Patient diary (patient-reported outcome)	Adult patients with non-infectious diarrhea, HIV-related	Not applicable
		Frequency of the watery bowel movement			

Disease/Condition	Indication and/or Claim(s) Description[32] [33]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Opioid induced constipation	Treatment of opioid-induced constipation	Frequency of spontaneous bowel movements without laxative use	Patient diary (patient-reported outcome)	Adult patients with opioid induced constipation	Not applicable
Opioid induced ileus, postoperative	Treatment of opioid induced ileus, postoperative	Resolution of opioid induced ileus, both the upper and lower gastrointestinal tract (i.e., toleration of solid food and first bowel movement)	Patient diary (patient-reported outcome)	Adult patients with opioid induced ileus, postoperative	Not applicable
Pancreas divisum undergoing endoscopic retrograde cholangiopancreatography (ERCP)	Facilitation of identification of the ampulla of Vater and the accessory papilla during ERCP	Cannulation of the minor duct of the pancreas	Clinician-reported outcome	Adult patients with pancreas divisum undergoing ERCP	Not applicable
Pompe disease, late-onset	Improvement in walking ability	Walking ability	6-Minute Walk Test (performance outcome)	Patients with Pompe disease, late-onset	Not applicable
Ulcerative colitis (UC)	Treatment of UC	Mucosal disease activity and severity	Endoscopy (clinician-reported outcome)	Adult and pediatric patients with UC	Not applicable
		Signs and symptoms of UC (blood and stool frequency)	Diary (patient-reported outcome)		
Ulcerative colitis (UC) (Pediatric)	To be determined	Signs and symptoms of pediatric UC	Modified Pediatric Ulcerative Colitis Activity Index (PUCAI) / TUMMY Scale (patient-reported outcome)	Pediatric UC in children 4-18 years of age with all disease activity states	Submitter: Shaare Zedek Medical Center (Israel) / SickKids Hospital (Canada) Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Ulcerative colitis (UC)	To be determined	Signs and symptoms of UC	Ulcerative Colitis-Patient Reported Outcomes Signs and Symptoms (UC-PRO/SS) (patient-reported outcome)	Adult patients with moderate to severe UC	Submitter: Evidera Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

BONE, REPRODUCTIVE, AND UROLOGIC PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[34] [35]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Benign prostatic hyperplasia (BPH)	Treatment of the signs and symptoms of BPH	Irritative (frequency, urgency, and nocturia) and obstructive (hesitancy, incomplete emptying, intermittency, and weak stream) symptoms	International prostate symptom score (IPSS) (patient-reported outcome)	Adult patients with BPH	Not applicable
		Strength of urine flow	Maximum urine flow rate (biomarker)		
Erectile dysfunction	Treatment of erectile dysfunction	Erectile function	Erectile function domain of the International Index of Erectile Function (patient-reported outcome)	Adult men diagnosed with erectile dysfunction	Not applicable
		Attainment of erection	Sexual encounter profile patient diary (patient-reported outcome)		
		Maintenance of erection			
Overactive bladder	Treatment of overactive bladder with symptoms of urge urinary incontinence, urgency, and urinary frequency	Number of incontinence episodes per 24 hours	Patient voiding diary (patient-reported outcome)	Patients with overactive bladder with symptoms of urge urinary incontinence, urgency, and urinary frequency	Not applicable
		Number of micturitions per 24 hours			
		Volume voided per micturition			

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[35] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[34] [35]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Vasomotor symptoms(VMS) Guidance for Industry: Estrogen and Estrogen/Progestin Drug Products to Treat Vasomotor Symptoms and Vulvar and Vaginal Atrophy Symptoms – Recommendations for Clinical Evaluation (draft)	Treatment of moderate to severe VMS (hot flashes/flushes) due to menopause	Frequency of VMS (hot flashes/flushes)	Patient diary (patient-reported outcome)	Postmenopausal women with moderate to severe VMS due to menopause	Not applicable
		Severity of VMS (hot flashes/flushes)	Daily severity score (patient-reported outcome)		
Vulvar and vaginal atrophy(VVA) Guidance for Industry: Estrogen and Estrogen/Progestin Drug Products to Treat Vasomotor Symptoms and Vulvar and Vaginal Atrophy Symptoms – Recommendations for Clinical Evaluation (draft)	Treatment of moderate to severe X (where X = vaginal dryness, dyspareunia, and/or vulvar/vaginal irritation/itching), a symptom of VVA due to menopause	Frequency of the most bothersome symptom (to the patient) of moderate to severe symptom of X (where X = vaginal dryness, dyspareunia (pain during intercourse), and/or vulvar/vaginal irritation/itching)	Patient diary (patient-reported outcome)	Postmenopausal women with moderate to severe symptoms of VVA due to menopause	Not applicable
		Vaginal maturation index (parabasal and superficial cells)	Laboratory measures (biomarkers)		
		Vaginal pH			

OFFICE OF HEMATOLOGY AND ONCOLOGY PRODUCTS (OHOP)

HEMATOLOGY PRODUCTS

Disease/Condition	Indication and/or Claim(s) Description[36] [37]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Chronic lymphocytic leukemia (CLL) Guidance for Industry: Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics	Treatment of CLL	Incidence of palpable hepatosplenomegaly	Composite of clinician-reported outcomes, patient-reported outcome, and laboratory/imaging measures (biomarkers) Note: B symptoms are assessed based on patient-reported outcome	Adult patients with CLL	Not applicable
		Size of lymph nodes; incidence of lymph nodes with nodularity			
		B symptoms evaluation (night sweats, fever, unexplained weight loss)			
		Laboratory measures (lymphocytes, neutrophils, platelets, histology)			
Cutaneous T-cell lymphoma (CTCL) Guidance for Industry: Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics	Treatment of CTCL	Skin involvement	Severity Weighted Assessment Tool in addition to other outcomes (e.g., response duration, time to progression, time to objective response) (clinician-reported outcome)	Adult patients with CTCL	Not applicable
		Physician's global assessing improvement or worsening in overall disease	7-Point Physician's Global Assessment (clinician-reported outcome)		
Deep vein thrombosis (DVT) and pulmonary embolism (PE)	Prophylaxis of DVT/PE	Incidence of venous thromboembolic events that includes deep vein thrombosis, non-fatal pulmonary embolism, and death due to thromboembolic in origin	Composite of thromboembolic events defined by a combination of biomarkers and clinician assessments (clinician-reported outcomes)	Adult patients at risk for DVT/PE	Not applicable

[36] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[37] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

Disease/Condition	Indication and/or Claim(s) Description[36] [37]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Mucositis (oral) due to hematologic malignancies receiving myelotoxic therapy requiring hematopoietic stem cell support	Decrease the incidence and duration of severe oral mucositis in patients with hematologic malignancies receiving myelotoxic therapy requiring hematopoietic stem cell support	Incidence and severity of oral mucositis (e.g., soreness/erythema, ulcers, oral intake tolerability)	World Health Organization Oral Mucositis Scale (patient-reported outcome)	Adult patients with hematologic malignancies receiving myelotoxic therapy requiring hematopoietic stem cell support	Not applicable
Myelofibrosis (MF) Guidance for Industry: Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics	To be determined	MF symptom severity	Patient reported outcome of MF symptom severity	Patients with intermediate or high risk MF, post polycythemia-vera MF and post-essential thrombocythemia MF who are symptomatic	Submitter: Critical Path Institute PRO Consortium's Myelofibrosis Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Paroxysmal Nocturnal Hemoglobinuria (PNH)	Reduction of fatigue	Disease-related fatigue	Functional Assessment of Chronic Illness Therapy - Fatigue (patient-reported outcome)	Adult patients with PNH and hemolysis requiring transfusion	Not applicable
Physical functioning in oncology	To be determined	Physical functioning	Patient Reported Outcome Measurement System (PROMIS) – Physical Function item bank (patient-reported outcome)	For use in clinical trials in oncology	Submitter: PROMIS Network Center Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

ONCOLOGY PRODUCTS 1

Disease/Condition	Indication and/or Claim(s) Description[38] [39]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Prostate cancer (metastatic castration-resistant) Guidance for Industry: Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics	Improvement in pain or delay in time to pain progression for patients with metastatic castration-resistant prostate cancer	Pain intensity	Brief Pain Inventory Item #3 – Short Form (patient-reported outcome)	Use in adult patients with metastatic castration-resistant prostate cancer along with other key oncology measures (e.g., overall survival)	Not applicable
Physical functioning in oncology	To be determined	Physical functioning	Patient Reported Outcome Measurement System (PROMIS) – Physical Function item bank (patient-reported outcome)	For use in clinical trials in oncology	Submitter: PROMIS Network Center Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[38] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[39] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.

ONCOLOGY PRODUCTS 2

Disease/Condition	Indication and/or Claim(s) Description[40] [41]	Outcome of Interest	COA (COA Type)	COA Context of Use	COA Qualification Information
Advanced non-small cell lung cancer (NSCLC) Guidance for Industry: Clinical Trial Endpoints for the Approval of Cancer Drugs and Biologics	To be determined	NSCLC symptoms	Non-Small Cell Lung Cancer - Symptom Assessment Questionnaire (NSCLC-SAQ) (patient-reported outcome)	To further describe a treatment benefit documented by tumor-based measures for adults with unresectable NSCLC	Submitter: Critical Path Institute PRO Consortium's Non-Small Cell Lung Cancer Working Group Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Plexiform neurofibromatosis 1 (PN1)	To be determined	Tumor-related pain intensity and tumor-related pain interference	Plexiform neurofibroma (PN) pain in children and adults (patient-reported outcome)	Children and adults with plexiform neurofibromatosis type 1 (NF-1)	Submitter: National Cancer Institute and National Institutes of Health Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information
Physical functioning in oncology	To be determined	Physical functioning	Patient Reported Outcome Measurement System (PROMIS) – Physical Function item bank (patient-reported outcome)	For use in clinical trials in oncology	Submitter: PROMIS Network Center Visit "Clinical Outcome Assessment Qualification Program Submissions" Web site for additional information

[40] Inclusion of a clinical outcome assessment in the COA Compendium is not intended to indicate that the measure is or should be the sole (or primary) determinant of a clinical benefit in a clinical trial.
[41] For specific indication and/or claim(s), please discuss with appropriate review division and when applicable, please refer to the referenced guidance document as appropriate.