

# 2024 IIS Strategic Priorities

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## **BODY CONTOURING: CoolSculpting® Elite, CoolTone®, Resonic®**

### **CoolSculpting® Elite**

1. To assess the impact of different treatment regimens using CoolSculpting® Elite alone or in sequence with other treatment modalities to understand factors that optimize patient selection, treatment, patient satisfaction and management.
2. To explore novel indications or use for CoolSculpting® Elite treatment including improving skin quality.

### **CoolTone®**

1. To assess the impact of different treatment regimens using CoolTone® alone or in sequence with other treatment modalities to understand factors that optimize patient selection, treatment (including maintenance), patient satisfaction and management.
2. To explore novel indications or use for CoolTone® treatment.

### **Resonic®**

1. To further understand the etiology and assessment of cellulite as well as cellulite presentation across diverse populations.
2. To assess the impact of different treatment regimens using Resonic® alone or in sequence with other treatment modalities to understand factors that optimize patient selection, treatment, patient satisfaction and management.
3. To assess the impact of Resonic® on skin remodeling and improving skin quality.
4. To explore novel indications or use for Resonic® treatment.

### **Things to consider for the investigator:**

- Limited funds may be available to support proposals.
- Support may be available for the development of manuscripts for independent research.

**FACIAL AESTHETICS:  
Botox/Vistabel, Fillers (Juvederm, Vycross, Hylacross), HArmonyCa  
Hybrid Filler, Pan Facial**

- The importance of treatment planning with aesthetic portfolio in establishing facial harmony/balance, and psychosocial impact as demonstrated on patient and observer reported outcomes.
- Benefit of adherence to a long-term treatment plan with the Allergan Aesthetics portfolio of products.
- Efficacy, safety, and psychosocial impact of multiple treatment areas with labelled dosage of Botox® over repeat treatment cycles.
- The importance of patient selection in delivering optimal outcomes (right patient/right product).
- Correlation of rheologic/physiochemical properties with clinical benefit/impact to patient.
- Innovative means to objectively measure clinical efficacy and psychosocial impact of treatment.
- Impact of treatment on skin architecture/skin quality
- Inclusivity in study populations
- Novel aesthetic uses of Allergan Aesthetic products

**Things to consider for the investigator:**

- Limited funds may be available to support proposals.
- Support may be available for the development of manuscripts for independent research.

# **PLASTICS AND REGENERATIVE MEDICINE: Breast Implants, ADMs, Fat Grafting**

## **Breast Implant Portfolio**

Enhancing scientific knowledge to improve clinical outcomes:

- Immediate & long-term patient outcomes with Natrelle® Inspira smooth implants and tissue expanders.
- Systemic symptoms reported by patients with breast implants (SSBI): etiology, pathogenesis, epidemiology, and management.
- BIA-ALCL/rare malignancies that have been reported in breast implant capsules: mitigation, etiology, pathogenesis, epidemiology, and treatments.

Supporting best practice techniques:

- Infection control techniques to improve patient outcomes (e.g. Keller funnel, aseptic technique)
- Keller funnel clinical outcomes measures
- Patient/implant matching for optimal outcomes (cohesivity matching)
- Global best practice surgical techniques to optimize outcomes with Allergan Aesthetics Breast Implant portfolio.
- Using Allergan Aesthetics breast surgical products to address clinical needs and optimize patient outcomes.

## **Regenerative Medicine Portfolio**

### **ADM Portfolio (Artia™, Alloderm™ and Strattice™)**

- Data demonstrating integration of ADM using histology and/or outcomes measures.
- Short- and long-term patient outcomes using Allergan Aesthetic ADMs for patients requiring soft tissue reinforcement or repair.
- Best practice surgical technique considerations to enhance patient outcomes.

### **Fat grafting benefits**

- Advance the understanding of surgical and patient outcomes using Revolve including patient/surgeon satisfaction, time savings and graft retention.

### **Things to consider for the investigator:**

- Limited funds may be available to support proposals.
- Support may be available for the development of manuscripts for independent research.

## **NEUROSCIENCE – MIGRAINE: OnabotulinumtoxinA/BoNT/A (BOTOX®), ubrogepant (Ubrovelvy), atogepant (Qulipta)**

### **Things to consider:**

- Studies will only be considered for compounds that have received regulatory approval for at least one indication in the country of interest.

### **Proposals for the topics listed below will NOT be considered:**

- OnabotulinumtoxinA vs placebo, vs other toxins, vs mAbs (monoclonal antibodies)
- Ubrogepant vs placebo, vs other acute treatments for migraine
- Atogepant vs placebo, vs other preventive treatments for migraine
- Double-blind, placebo controlled pediatric studies
- Placebo controlled studies for treatment during premonitory/prodrome phase

### **Priority will be given to proposals investigating the following areas:**

#### **Migraine Disease State**

- Characterize and describe disease state across migraine
- Impact of disease management on disease state including acute and preventive treatment patterns
- Impact of early intervention of disease
- Impact of COVID, COVID vaccines and COVID-related treatments on disease outcomes in patients treated with onabotulinumtoxinA, ubrogepant and/or atogepant

#### **OnabotulinumtoxinA/BoNT/A (BOTOX®) - Chronic Migraine**

- Further understand the mechanism of action of onabotA on sensory pathways linked to migraine.
- Preclinical research to understand potential synergistic effect of onabotA used in combination with mAbs/gepants
- Understanding how onabotA can improve the lives of patients with migraine who have failed prior treatment with CGRP mAbs.
- Real-world effectiveness of the combination treatment of adding a CGRP mAb/gepant to a patient with chronic and/or high frequency episodic migraine being treated with onabotA and vice-versa
- Understanding the impact of onabotA on migraine associated co-morbidities, including the prevention of associated comorbidities.
- Safety and effectiveness of onabotA treatment in other primary or secondary (HA) disorders
- Understand the impact of onabotA on functional outcomes, quality of life, health resource utilization and work productivity/return to daily activities
- Assess the long-term real-world economics and effectiveness of onabotA.
- Real-world evidence demonstrating medico-economic impact of delaying treatment of patients with chronic or high frequency episodic migraine due to access limitations.
- Understanding the impact of onabotA on chronic or high frequency episodic migraine (using PREEMPT) in patients with concomitant chronic conditions requiring additional injection paradigm (ie. peri-orbital pain, allodynia, cervical dystonia).

**Ubrogepant (Ubrelvy) - Acute Treatment of Migraine**

- Safety and effectiveness of Ubrogepant + other acute treatments
- Safety and effectiveness of Ubrogepant + preventive treatments for migraine
- Impact of Ubrogepant management on disease state
- Impact of Ubrogepant use and Medication Overuse Headache (MOH)

**Atogepant (Qulipta) - Migraine Prevention**

- Safety and effectiveness of Atogepant + Ubrogepant
- Safety and effectiveness of Atogepant + OnabotulinumtoxinA
- Safety and effectiveness of Atogepant + other migraine treatments
- Treatment switching
- Impact on ictal/interictal QoL and non-HA symptoms (including function, daily activities, etc.), work productivity and/or cognition
- Impact on co-morbidities
- Impact on health care resource utilization
- Impact of Atogepant management on disease state

## **NEUROSCIENCE – NEUROTOXIN THERAPEUTICS: Botox (OnabotulinumtoxinA)**

For the purposes of this document the IIS Strategic Priorities will be described for these indications:

- Toxin Science and New Indications
- Spasticity and Movement Disorders (focus on adult spasticity and cervical dystonia)
- Urology (focus on OAB and NDO)

For Migraine and for Botox Cosmetics indications, please refer to the applicable sections

### **Things to consider:**

- Limited funds may be available to support proposals

### **Proposals for the topics listed below will NOT be considered:**

- Studies conflicting with current research and development programs and strategies
- For Spasticity - Assessment of anticoagulants pre/post injection procedure
- For SMD - Imaging studies with no clear patient reported outcomes
- For Urology – Evaluation of Botox for Premature Ejaculation (PE) or Benign Prostatic Hypertrophy (BPH)

### **Priority will be given to proposals investigating the following areas:**

#### **Toxin Science and Novel Indications**

- Real world evidence of clinical and health economic outcomes of Botox utilization versus other toxins, including the consequences of toxin switching
- Botox utilization to broad dosing protocols in multi-indication patients
- Novel indications, applications and treatment paradigms in alignment with areas of company focus.
- Pain, sensory, anti-inflammatory mechanisms of action of Botox
- Non-classical mechanism of action of Botox
- Preclinical studies assessing Botox versus other toxin

#### **Spasticity**

- Botox for treatment of pain associated with spasticity
- Studies assessing the applicability of tools and biomarkers as a measure of efficacy and/or predictors for early spasticity diagnosis
- Studies that assess pharmacodynamics of Botox in relation to the mechanism of action (course of action)
- Novel outcome measures to recognize, diagnose and assess spasticity Impact of early diagnosis and intervention on overall outcomes when treated with Botox
- Impact of treatment adherence/discontinuation
- Real world safety, efficacy and utilization of Botox (assessing muscles, doses and retreatment)
- Impact of Botox treatment on comorbidities (e.g., depression/anxiety)
- Impact of Botox on patient relevant outcomes (including but not limited to patient satisfaction, functional impairment, pain relief, quality of life etc.)

- Assessment of improvement of function and/or specific functional goal attainment after treatment with Botox in spasticity due to any etiology (beyond post stroke etiology)
- Optimization of Botox therapy with pattern-based treatment paradigms
- Optimization of Botox when used in combination with other therapies
- Optimization of Botox therapy with utilization of new technologies, training tools and/or treatment paradigms
- Impact of Botox when used alone or in conjunction with other therapies during rehabilitation

### **Movement Disorders**

- Botox for treatment of pain associated with movement disorder (focus on cervical dystonia)
- Clinical and/or Pharmacoeconomic impact of early diagnosis and intervention
- Studies assessing approaches/predictors for prompt diagnosis and early intervention with Botox for Cervical Dystonia to obtain optimized patient outcomes
- Real world observations describing impact of Botox on comorbidities (e.g., depression/anxiety, migraine) and/or patient reported outcomes (including but not limited to patient/physician satisfaction, functional impairment, pain relief, quality of life etc.)
- Optimization of Botox therapy with utilization of new technologies, training tools and/or treatment paradigms
- Studies that assess pharmacodynamics and/or clinically meaningful duration of Botox effects
- Impact of treatment adherence/discontinuation on pharmacoeconomic and functional outcomes
- Studies that can help with the disease classification in relation to essential tremor
- Botox for treatment and optimizing dosing paradigm in relation to essential tremor
- Studies focusing on the treatment goals and patient outcomes associated with essential tremor

### **Urology Indications**

- Studies to assess long-term real-world economics, safety and/or effectiveness of Botox in OAB and/or NDO
- Mechanism of action of Botox in the bladder for overactive bladder conditions or painful pelvic conditions
- Assessment of treatment paradigms (e.g., injection number or pattern), treatment administration setting, and other optimizations, which could impact tolerability, treatment adherence and/or patient satisfaction
- Studies to assess variables that impact time between diagnosis and advancement to third line therapies (including prolonged cycling on oral medications)
- Impact of treatment adherence/discontinuation when utilizing Botox on pharmacoeconomic and functional outcomes
- Studies showing benefit of Botox specifically in men with OAB/NDO or other novel applications



## **NEUROSCIENCE – PARKINSON’S DISEASE: Duopa/Duodopa, Produodopa**

### **Things to consider:**

- Studies will only be considered for compounds that have received regulatory approval for at least one indication in the country of interest.

### **Proposals for the topics listed below will NOT be considered:**

- Head-to-head proposals between device aided therapies

### **Priority will be given to proposals investigating the following areas:**

#### **Parkinson’s Disease**

- Disease burden/Progression:
  - Further understand the characteristics and/or burden in different stages of advancing Parkinson’s disease (PD) inadequately controlled by optimized oral therapy including but not limited to the impact on: nighttime sleep disturbances, patients, caregivers, and healthcare resource utilization
- Disease Management:
  - Clinical utility of diagnostics or technology to identify signs and symptoms of Advancing Parkinson’s Disease and resultant changes in care.
  - Understand the right time for intervention (and provide referral, if needed) using different tools (i.e., Tools/Criteria such as “5-2-1”, MyPDCare, and MANAGE-PD; biomarkers, technology based objective measures (TOM) for use by HCP, patient/caregiver, and patient foundations/associations.
  - Challenges with current treatment options
- Patient Perspective:
  - Evaluate patient barriers to refusal or initiation of invasive treatment (with a focus on subcutaneous infusion options) despite insufficient symptom control

#### **Continuous dopaminergic stimulation (CDS)**

- Short and long-term effects of CDS vs pulsatile stimulation, including but not limited to use in earlier stages of PD to assess the ability of continuous therapies for disease modification and prevention/delay of motor complications
- Pharmacological effects of CDS over 24 hrs.
- Investigate indicators/markers (including but not limited to laboratory, clinical, imaging) to provide evidence to support whether continuous drug delivery translates to continuous dopaminergic stimulation in patients.
- Evaluate preclinical impact of CDS on neuroinflammation

#### **Levodopa Carbidopa Intestinal Gel/Carbidopa Levodopa Enteral Suspension (LCIG/CLES)**

- Evaluate LCIG/CLES efficacy and safety in subpopulations of advanced PD (APD) patients uncontrolled on orals where currently limited data exists
- Understanding the use of LCIG/CLES in combination with or after failure with other device aided therapies (DATs)
- Further understand the characteristics of responders to LCIG/CLES therapy
- Additional areas:
  - Evaluate LCIG/CLES in other Parkinsonism disorders

## **Produodopa**

- Evaluate Produodopa efficacy and safety in subpopulations of advanced PD (APD) patients uncontrolled on orals including, but not limited to: age <65 years, Body-Mass-Index subgroups (eg BMI <18.5, 18.5-25, >25), cognitively impaired, treated with DATs previously, patients with impulse control disorders (ICDs), difficult to treat dyskinesia, freezing of gait, difficult to treat prominent non motor symptoms (NMS), axial symptoms in whom oral treatments are no longer effective and underserved populations/diverse ethnicities/race
- Evaluate device-aided therapy sequencing patterns
- Evaluate efficacy and safety in PD patients earlier within “advanced” PD (eg, time to motor fluctuations < 5 years, less daily motor complication burden, shorter disease duration), “active” patients (e.g., with regards to exercise levels)
- Characterize APD medication management to achieve monotherapy with Produodopa, including pill burden reduction
- Further understand the characteristics of Produodopa patient population and responders, clinical assessment tools, and key clinical and/or health economic outcomes, including but not limited to:
  - Patient and caregiver-related outcomes, preferences, and satisfaction (patient/caregiver experience), activities of daily living (ADL), quality of life (QoL), perceived independence
  - General or specific Non-Motor Symptoms (NMS) including but not limited to sleep, apathy, fatigue, pain
  - Morning akinesia or dystonia
  - Using Technology based Objective Measurements (e.g., wearable devices, biosensors, and others) as surrogates of efficacy and safety of Produodopa in APD patients
- Long-term data on effectiveness and safety (including infusion site events), factors leading to discontinuations and interventions to improve adherence.
- Understand titration and the impact of different settings for the treatment initiation with Produodopa, evaluated by patient/Caregiver-related outcomes (like satisfaction, self-efficacy, QoL, ADL) in APD, under the following circumstances
  - Outpatient versus inpatient titration setting (or even fully remote initiation, see telemedicine)
  - General Neurologists (GN)-initiated versus Movement Disorder Specialist (MDS)-initiated titration
  - Different nurse care models
  - Use of telemedicine
- Understand practical aspects of long-term therapy, including evaluating handling of the infusion device (e.g., infusion site rotation, alternative infusion sites, frequency of needle placement, use of different infusion sets), skin care regimens for the prevention or treatment of skin reactions and nodules etc., nighttime dosing
- Understand impact of 24 hrs delivery on lifestyle and societal outcomes (e.g. return to activity, lifestyle, etc)
- Additional focus areas:
  - Evaluate Produodopa in other Parkinsonian Syndromes.
  - Evaluation of Biomarkers in APD patients and response when treated with Produodopa

## **NEUROSCIENCE – PSYCHIATRY – VRAYLAR (CARIPRAZINE): Bipolar Disorder I (BP-I) depressive, manic, and mixed episodes; Schizophrenia; and US only: Adjunctive treatment of MDD (aMDD)**

Cariprazine (VRAYLAR) is an orally active atypical antipsychotic. It is a partial agonist at central dopamine D3/D2 and serotonin 5-HT1A receptors and has antagonist activity at serotonin 5-HT2A receptors.

We welcome proposals from Investigators from under-represented groups.

Vraylar is approved for adults with:

- Bipolar Disorder I (BP-I) - depressive, manic, and mixed episodes
- Schizophrenia
- US only: Adjunctive treatment of MDD (aMDD)

Studies will only be considered for compounds that have received regulatory approval for at least one indication in the country of interest.

### **Priority will be given to proposals to investigate the following areas:**

- Investigation of disease states where Vraylar's pharmacology matches with the hypothesized underlying biology of psychiatric illnesses.
- Understanding the impact of Vraylar on the following in BP-I and MDD populations:
  - Sexual functioning
- Additional areas of interest for the approved indications (BP-1, schizophrenia, and MDD) include:
  - Increasing accuracy of BP-I Disorder diagnosis
  - Real world experience with and implementation of Rapid Mood Screener
  - Variance in the response to Vraylar based on the status of the gut microbiome.
  - Burden of disease
  - Novel outcomes related to treatment with Vraylar
  - Efficacy on anhedonia as an adjunctive treatment for MDD
  - Long-term efficacy data as adjunctive therapy for MDD
  - Efficacy and safety in patients with substance use disorders
  - Potential to improve cognitive deficits in patients
  - Treatment with cariprazine in first episode psychosis
  - Combination treatment with cariprazine in schizophrenia and bipolar-I disorder

### **Proposals in the following areas will NOT be considered:**

- Indications for which Cariprazine (VRAYLAR) has been or is being evaluated:
  - Pediatric adjunctive Major Depressive Disorder (aMDD)
  - Pediatric BP-I disorders
  - Pediatric schizophrenia
  - Maintenance treatment of BP-I disorder in adults
  - Irritability associated with autism spectrum disorder (ASD)
  - Negative symptoms in Schizophrenia
- Populations at high risk for impulse control dysfunction/compulsive behavior
- Patients at high risk for seizures
- Populations and/or indications under consideration by AbbVie

## **EYE CARE:**

### **DURYSTA (bimatoprost intracameral implant); REFRESH Portfolio of Artificial Tears, XEN 45/63, Ozurdex**

#### **Durysta**

- Real World Evidence (RWE) on effectiveness and safety of Durysta
- Risk factors leading to corneal adverse and methods to decrease such events with Durysta (e.g., anatomic profile, placement, etc.)
- Identification of predictors of long-term Intraocular Pressure (IOP) lowering effect with Durysta
- Impact on topical AEs profile (including Ocular Surface Disease (OSD)) of switch from drops to Durysta
- Impact of Durysta on adjunctive medication use beyond PGA drops
- Effectiveness and transition to subsequent treatments after Durysta
- Effectiveness of Durysta after Selective Laser Trabeculoplasty (SLT)
- Effectiveness of Durysta before and/or after prior interventions (e.g., Glaucoma Surgeries)
- QoL, patient and ECP satisfaction surveys

#### **Refresh Artificial Tear Portfolio**

- Quantification of the duration of relief of symptoms with Refresh artificial tears use
- Effectiveness of combination of Refresh products
- Real world outcomes in aqueous deficient, mixed, and evaporative dry eye disease (i.e. meibomian gland dysfunction) patient subtypes with the use of Refresh artificial tears
- Real world outcomes of patients with dry eye symptoms due to digital device use with lipid-containing Refresh artificial tears
- Real world outcomes related to preservative-free Refresh artificial tears

#### **Refresh Digital**

- Real world outcomes of patients with eye dryness due to digital device use while using lipid-containing Refresh artificial tears

#### **XEN 45/63**

- Improvement of patient outcomes via novel surgical techniques
- Data after failure of other MIGS procedures
- Data comparing with other bleb forming procedures (eg Trabeculectomy, Preserflo) or with Tube shunts
- Trabeculectomy or repeat XEN after previously failed XEN surgery.
- Rate of Progression in Glaucoma and Visual Field forecasting algorithm
- Study when implanted earlier in treatment paradigm (e.g. after 2 drops)
- Impact of COVID 19 on Glaucoma progression and IOP control
- Data in Angle closure glaucoma

## Ozurdex

- Biomarkers in DME and RVO: Identifying and validating predictors of inflammation or Ozurdex therapeutic benefit (with or without AI).
- Use of Ozurdex in phakic patients with DME
  - First and second line use of Ozurdex
- Sequential use of Ozurdex and Anti-Vascular Endothelial Growth Factor (a-VEGF) (e.g. Ozurdex as 1<sup>st</sup> line).
- Safety profile of Ozurdex in relationship to shorter or extended treatment intervals"
- Efficacy and safety of Ozurdex across indications (e.g. DME, RVO, Uveitis) in 1<sup>st</sup> line.
- AI's role in diagnosing, classifying, monitoring, and/or treating DME, RVO, and/or Uveitis.
- Limitations of standards of care in DME
  - e.g. focusing on functional and anatomical outcomes switching between different a-VEGF agents
  - e.g. identifying patient sub-groups/clinical characteristics where limitations in aVEGF standard of care are more pronounced.
- RWE in patients with suboptimal response to anti-VEGF therapy in DME/RVO.
- Combination use of Ozurdex and anti VEGF.

## **IMMUNOLOGY – GENERAL: Adalimumab (Humira), Risankizumab (Skyrizi), Upadacitinib (Rinvoq)**

Pre-clinical and clinical proposals for diseases with high unmet need/burden, few to no suitable approved therapeutic options and strong rationale for target engagement in causality of disease may be submitted for consideration.

Clinical interventional studies will only be considered for compounds that have regulatory approval for use in at least one indication in that country.

For preclinical studies utilizing upadacitinib and risankizumab outside of approved indications only *ex-vivo*, *in-vitro* or *in-silico* studies will be considered.

Studies documenting treatment patterns and outcomes in under-represented populations.

Studies utilizing adalimumab will be considered only for investigating conditions without suitable alternative treatment options, where sufficient evidence exists to support a hypothesis for life-altering outcomes.

## **IMMUNOLOGY – GASTROENTEROLOGY Risankizumab (Skyrizi) & Upadacitinib (Rinvoq)**

1. Prevention and treatment of different subsets of IBD, including but not limited to Acute Severe Ulcerative Colitis, pouchitis, ileal disease, fistulizing and stricturing disease, and post-operative recurrence in Crohn's disease
2. Further the understanding of mechanisms of diseases in IBD, predictive and prognostic factors of disease severity and progression, response to therapy
3. Optimal monitoring of disease, including symptoms and inflammation in IBD, non-invasive mucosal healing monitoring tools (e.g., ultrasound, MRI) and proposals of validation of monitoring tools (e.g., AI of endoscopic scoring)
4. Broadening and exploration of drug mechanisms, including JAK and IL-23, evaluation of biomarkers geared towards identifying different drivers of response, targeted therapy approaches and developing and testing patient stratification hypotheses.
5. Broadening the knowledge on early and sustained control of inflammation in IBD to prevent disease worsening, adverse disease outcomes or optimize long term outcomes
6. Optimizing strategies to mitigate the holistic burden of IBD (total cost of illness, quality of life, co-morbidities, unmet needs) with special focus on underserved populations
7. Understanding of dosing and sequence strategies to optimize disease control

## **IMMUNOLOGY – DERMATOLOGY: Adalimumab (Humira), Risankizumab (Skyrizi), Upadacitinib (Rinvoq)**

### **Overarching**

**Indications: PsO, PsA, AD, HS, Vitiligo, AA**

**Priority consideration will be given to applications proposing to investigate the following areas of interest:**

1. Impact of treat-to-target strategies, treatment goals and initiatives to advance quality of care in managing disease
2. Understanding treatment patterns, including dosing, sequencing, and compliance, including persistence, adherence, adverse events, and outcomes in diverse populations including patients with skin of color
3. Burden of disease, including co-morbidities, psychosocial impact (stigma, mental health), access to care, and health care resource utilization (HCRU), as well as the cumulative long-term impact of disease across varying patient types (age, extent of disease, skin phototype)
4. Disease prevalence and natural course of disease, including progression
5. Disease pathogenesis

### **Indication: Psoriatic Disease (PsO, PsA)**

**Compound: Risankizumab (Skyrizi)**

6. Evaluation of the impact of Psoriatic Disease on the Cumulative Life Course Impairment (CLCI)
7. Identification of predictors of early and durable response with Risankizumab
8. Evaluate the impact of Risankizumab in specific understudied sub-populations and on Psoriatic-associated comorbidities (i.e., PsA, metabolic syndrome)
9. Real-world effectiveness and treatment patterns of Risankizumab in Psoriatic Arthritis

### **Indication: Atopic Dermatitis (AD)**

**Compound: Upadacitinib (Rinvoq)**

10. Effectiveness and safety of Upadacitinib in AD, in specific understudied sub-populations
11. Understanding biomarkers of systemic inflammatory response in Atopic Dermatitis and how these may impact therapeutic response.
12. Understanding the importance, implementation and/or validation of optimal disease control (e.g., minimal disease activity, EASI 90, NRS 0/1) and its impact on patients
13. Impact of Upadacitinib on AD co-morbidities

### **Indication: Hidradenitis Suppurativa (HS)**

14. Practical diagnostic, assessment, monitoring and imaging tools that aid in disease classification, assessment of disease severity, activity progression, flares, response to treatment (including higher level efficacy)
15. Patient types/segments, including the role of specific cytokine pathways (IL-1, JAK), as well as their needs with respect to disease management
16. Impact of early referral, diagnosis and treatment in HS including impact on progression, and the consequences of underdiagnosed / undertreated disease

**Indication: Vitiligo**

17. Disease scoring and assessment measures, including QoL measures and characterization of disease severity beyond the extent of lesions (e.g., patient impact)

**Indication: Alopecia Areata (AA)**

18. Disease scoring and assessment measures, including QoL measures, and quality of hair growth

**Other Inflammatory Skin Diseases**

19. Research to understand the pathogenesis, disease course, burden (including co-morbidities, CLCI), disease assessments and, treatment goals and patterns of other inflammatory skin diseases
20. Efficacy/effectiveness and safety of approved AbbVie assets in other inflammatory skin diseases



# IMMUNOLOGY – RHEUMATOLOGY:

## Rinvoq (Upadacitinib-UPA) and Skyrizi (Risankizumab-RISA)

### Rheumatology / Overarching

1. Use of JAKi and/or IL-23i in inflammatory disease related to but not limited to adherence, persistence, treatment patterns/strategies and comparative effectiveness (observational or interventional)
2. Biomarkers predictive of response to treatment and/or prognostic of disease progression
3. Outcomes associated with patients achieving and/or maintaining stringent disease control
4. Addressing elements of standard of care, specifically, early and appropriate diagnosis, treatment targets, healthcare disparities, workplace shortages and understanding the barriers to implementing goal directed therapy
5. Effectiveness of switching to JAKi or IL-23i, versus cycling through the same previous MOA (e.g., in patients with lack of response/intolerance to TNFi switching to a JAKi vs another TNFi)

### **Compound:** Rinvoq (Upadacitinib-UPA)

#### Indication: UPA in GCA/TAK

1. Understanding the burden of GC treatment and characterize the unmet need in GCA/TAK Role of the JAK/STAT pathway in the pathogenesis of large vessel vasculitis
2. Characterizing patients not responding to current therapies (e.g., IL-6)
3. Impact of therapies in vascular tissue damage (imaging / biopsy studies)

#### Indication: UPA in SLE

1. Understanding pathogenic mechanism involving JAK/STAT pathway in Cutaneous lupus Erythematosus (CLE), Lupus Nephritis (LN) and Neuropsychiatric SLE (NPSLE)
2. Understanding what Lupus Arthritis is (e.g., MRI and/or ultrasound imaging findings) and the role of JAK/STAT pathway. Characterization of joint involvement in SLE, including the role of JAK/STAT, disease progression and disease monitoring (MRI/Ultrasound etc.)
3. Understanding patient-reported neuropsychiatric symptoms (e.g., brain fog, cognitive dysfunction, fatigue, etc.) in SLE and the role of JAK/STAT pathway
4. Identification of SLE endotypes, patients susceptible to flare, and gene signatures, soluble factors, or other biomarkers that can predict early response to treatment and/or characterize patients who will not respond to current treatment modalities

#### Indication: UPA in axSpA

1. Real world impact of upadacitinib on core axSpA domains with a focus on stringent disease activity, pain, functioning, and structural damage
2. Impact of upadacitinib on extra-musculoskeletal manifestations across the axSpA spectrum
3. Use of imaging modalities to assess the effectiveness of upadacitinib across the axSpA spectrum

**Indication: UPA in PsA**

1. Real world impact of upadacitinib treatment on PsA domains (effectiveness including patient reported outcomes, safety and tolerability)
2. Impact of upadacitinib treatment on imaging confirmed axial PsA
3. Impact of upadacitinib on extra-musculoskeletal manifestations in PsA

**Compound: Skyrizi (Risankizumab-RISA)**

**Indication: Risa in PsA**

1. Impact of risankizumab treatment on PsA domains and related conditions with a focus on musculoskeletal symptoms, axial manifestations, and structural joint damage
2. Adherence and persistence with risankizumab in PsA
3. Understanding novel treatment strategies involving risankizumab

## ONCOLOGY:

AbbVie Oncology compounds with areas of interest for investigator-initiated studies are listed below. AbbVie is accepting preclinical and clinical applications in 2024.

**Below you will find overarching guidance as it relates to all Oncology compounds included in this list. In addition to the overall 'Oncology' guidance and priorities outlined below, there are compound-specific areas of priority as listed in each asset sub-section.**

Priority will be given to applications proposing to investigate the following areas:

- Novel therapeutic combinations
- Evaluation of mechanisms to overcome resistance
- Evaluation of novel and additional clinical endpoints and/or exploratory predictive models or biomarkers

Things to consider for the investigator:

- Prior to Regulatory approval, a clinical interventional IIS may be considered only after the safety profile has been established for a compound.
- AbbVie's medical science liaisons are available to provide guidance throughout this process.
- Funding of research must not exceed local fair market value, nor be used for expenses not associated with the conduct of the research.
- Submissions that include populations that duplicate AbbVie's clinical development are not a high priority.

In addition to our areas of interest for oncology IIS, we have a diverse clinical trial program. Details of our AbbVie oncology pipeline can be found at this [link](#).

### **Venetoclax (ABT-199)**

Venetoclax is a BCL2 inhibitor with scientific rationale for evaluation across a broad variety of hematologic malignancies/disorders including, but not limited to chronic lymphocytic leukemia (CLL) and acute myeloid leukemia.

The IIS Strategic Priorities for Venetoclax are aligned to the overarching guidance provided above which outlines the oncology areas of interest.

Notable considerations for the investigator:

- Adult solid tumor proposals will not be accepted.

### **Epcoritamab**

Epcoritamab is a CD3xCD20 bispecific antibody with scientific rationale for evaluation in B-cell Malignancies.

Epcoritamab is being investigated in a number of hematological indications, including but not limited to: diffuse large B-cell lymphoma (DLBCL), follicular lymphoma (FL), chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL) and marginal zone lymphoma (MZL).

In addition to the overall 'Oncology' guidance and priorities outlined above, priority will be given to Epcoritamab applications proposing to investigate the following areas:

- Evaluation of Epcoritamab management in various patient care settings
- Evaluation of real-world data including but not limited to treatment patterns and patient-reported outcomes

Guidance for Pre-clinical proposals:

Pre-clinical and translational applications will be reviewed to prioritize proposals aligned to the following goals:

- Investigate rationale for combinations that improve efficacy, safety and synergize with Epcoritamab's mechanism of action, using relevant models
- Evaluate relationship between Epcoritamab and the tumor immune micro-environment
- Evaluate efficacy of Epcoritamab in patient-derived samples for indications of particular interest, including but not limited to Richter's Transformation, MCL, post-CAR-T, high risk DLBCL and FL

**ABBV-383**

ABBV-383 is a BCMAxCD3 bispecific T-Cell Engager with scientific rationale for evaluation in Multiple Myeloma and other Plasma Cell Dyscrasias (PCDs).

In addition to the overall 'Oncology' guidance and priorities outlined above, priority will be given to ABBV-383 applications proposing to investigate the following areas:

- Novel therapeutic combinations in Multiple Myeloma and other PCDs
- Exploration in special patient populations
- "Response adaptive and MRD guided approaches" OR "Patient-centric trial designs"
- Clinical indications where there is scientific rationale outside of Multiple Myeloma

Notable considerations for the investigator:

- Solid tumor proposals will not be accepted
- Low risk / Intermediate Risk SMM; MGUS proposals will not be accepted
- PK studies will not be accepted

Guidance for Pre-clinical proposals:

Pre-clinical and translational applications will be reviewed to prioritize proposals aligned to the following goals:

- Provide rationale for combinations that improve efficacy, safety and synergize with ABBV-383 MOA
- Evaluate relationship between ABBV-383 and the tumor immune microenvironment
- Evaluate novel combinations and mechanistic studies in relevant models

**Telisotuzumab vedotin (Teliso-V, ABBV-399)**

Teliso-V is an antibody drug conjugate (ADC) targeting c-Met protein overexpression with scientific rationale for evaluation in non-small cell lung cancer and other solid tumor malignancies.

In addition to the overall 'Oncology' guidance and priorities outlined above, priority will be given to Teliso-V applications proposing to investigate the following areas:

- Clinical proposals evaluating Teliso-V based novel combinations (e.g; IO, TKI, RT or others) for NSCLC non-squamous cMET OE tumors based on robust preclinical evidence demonstrating combinatorial efficacy.
- Evaluate potential Teliso-V based combinations (IO and others) with the rationale for extrapolating treatment strategies to NSCLC patients with unmet needs (e.g., brain metastases).
- Proposals assessing activity of Teliso-V (either as monotherapy or in combination) in earlier lines of therapy for NSCLC non-squamous cMET OE tumors.

Notable considerations for the investigator:

- Pediatric proposals will not be accepted.
- Proposals with squamous NSCLC histology will not be accepted.
- Monotherapy proposals in NSCLC patients with EGFR actionable mutations will not be accepted.
- Proposals with translational/biomarker components will be assessed on a case-by-case basis based on unmet scientific questions.

For non-clinical IIS:

- Evaluate MET contribution to NSCLC disease biology and evolution in emerging therapeutic options
- Relationship and crosstalk between MET aberrations (expression/amplification/mutation) and the tumor immune microenvironment
- Concordance of c-Met protein overexpression between biopsy, cytological samples and circulating tumor cells
- Alternate emerging technological modalities for protein overexpression measurement
- Effect of Teliso-V on the tumor immune microenvironment
- IHC platform and antibody comparison for c-Met protein overexpression
- Overlap between c-Met protein overexpression and oncogenic and non-oncogenic NSCLC biomarkers

### **Navitoclax (ABT-263)**

Navitoclax (ABT-263) is a BCL-XL/BCL-2 inhibitor with scientific rationale for evaluation across several hematologic malignancies, particularly myeloproliferative neoplasms (MPN).

In addition to the overall 'Oncology' guidance and priorities outlined above, priority will be given to Navitoclax applications proposing to investigate the following areas within MPN:

- Exploration of navitoclax combination therapy in special patient populations

Notable considerations for the investigator:

- Clinical indications other than MPN are of lower priority.
- Solid tumor proposals will not be accepted.

Areas of interest for non-clinical/preclinical IISs:

- Evaluating preclinical efficacy and correlative effects of BCL-XL inhibition in myeloproliferative neoplasms (MPN).
- Apoptosis research (e.g., senescence, fibrosis, immune cells).

## **Specialty – HIV: Pathway to HIV Cure**

AbbVie is committed to developing new treatments which provide virologic control off-ART with the long-term goal of regimens that can induce an HIV cure.

In this context AbbVie is interested in proposals covering the following areas:

1. Biomarkers in support of HIV virologic control and possibilities for cure, including but not limited to proof-of-mechanism biomarkers, new methods of viral load monitoring, predictors of response, predictors of viral rebound and changes in the HIV reservoir.
2. HIV+ population-specific studies and methodologies for ART-free virologic control and/or cure research, including but not limited to new MoAs or combinations, treatment experienced, persistent low viremia, adolescents, paediatrics, and PLWH with diverse socioeconomic backgrounds.

## Specialty – Hepatology – Maviret/Mavyret: Hepatitis C Virus (HCV)

AbbVie is committed to support global efforts to meet WHO target of HCV elimination as a major public health threat by 2030.

In this context, AbbVie is interested in scientific study proposals addressing any of the following priority areas:

1. Research aiming to explore sustainable solutions and models, including 8-week treatment, to allow simplification of the HCV care continuum and/or accelerate the path to elimination, in the following populations\*: A) high incidence of HCV, B) high risk of HCV transmission, C) sub-optimal linkage to care and D) patients receiving concomitant medications of interest including those with substance use disorders. Examination of models that successfully incorporate non-liver specialists into the HCV care, include but are not limited to addiction specialists, OB/GYN, psychiatrist, nurses, and primary care physicians. E) Real-world implementation of test and treat model including but not limited to the assessment of chronicity

\*Patient populations may include people who inject drugs (PWID), immigrants, incarcerated, mental health patients

2. Clinical and economic outcomes of 8-week treatment in patients with acute and recently acquired HCV to enable further simplification of HCV care across disease stages.
3. Epidemiological research in HCV populations of interest (e.g., PWID, immigrants from high-prevalence countries, incarcerated, mental health patients, geriatric patients, pregnant persons, including pediatric transmission).

## Endo-Metabolic: CREON

Abbvie is interested in scientific study proposals that would address the following priority clinical areas:

1. Burden of exocrine pancreatic insufficiency (EPI) disease and impact of treating EPI in:
  - a. Acute Pancreatitis
  - b. Celiac Disease
  - c. GI surgeries that could result in EPI
  - d. Fatty pancreas
  - e. Diabetes mellitus type 1, or 2
2. Outcomes in EPI patients treated with CREON® (pancrelipase) with underlying conditions of EPI, excluding chronic pancreatitis in adults
3. Novel approaches (clinical tools, biomarkers, devices) that
  - a. Accelerate/ease the diagnosis of EPI
  - b. Improve adequate pancreatic enzyme replacement therapy (PERT) dosing
  - c. Improve PERT adherence
  - d. Assess outcome of PERT treatment beyond stool fat measurements



**ANTI-INFECTIVES:  
Avycaz (ceftazidime/avibactam), Teflaro (ceftaroline fosamil)**

**Key Focus Areas: Avycaz (ceftazidime/avibactam)**

- Comparative clinical outcome data in infections caused by carbapenem-resistant Enterobacterales (CRE) and multi-drug resistant (MDR) *Pseudomonas aeruginosa*
- Studies (pre-clinical and clinical) evaluating emergence of resistance of Gram-negative bacteria, including prevalence of resistance, mechanisms of resistance, and strategies for prevention
- Clinical outcomes in special populations treated with CAZ-AVI (i.e., immune-compromised, transplant)

**AbbVie is interested in scientific study proposals requesting product only addressing any of the following priority areas for Teflaro (ceftaroline fosamil):**

**Key Focus Areas: Teflaro (ceftaroline fosamil)**

- Comparative time to clearance data of *S. aureus* bacteremia vs standard of care (vancomycin, daptomycin)
- Dose optimization and/or outcomes in difficult to treat infections, including but not limited to methicillin resistant *S. aureus* (MRSA) bacteremia and MRSA pneumonia

## **GI CARE: Linaclotide**

AbbVie is interested in scientific study proposals that would address the following priority areas:

1. Efficacy and safety of linaclotide to treat pediatric patients with constipation, including alternative doses, special patient populations, and in real-world settings
2. Clinical and/or real-world use evaluating efficacy and safety of linaclotide to treat additional adult patient populations suffering from constipation and/or abdominal symptoms
3. Novel devices, clinical tools and/or biomarkers that:
  - a. Increase the ease of confident diagnosis of IBS-C and CIC
  - b. Improve patient clinical experience, outcomes and/or adherence to linaclotide treatment
  - c. Assess novel outcomes of IBS-C and CIC treatment