2018 IIS Strategic Priorities

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Hepatology - Compound: Glecaprevir and Pibrentasvir

AbbVie is particularly interested in scientific study proposals which would address one of the following priority areas:

1. To explore novel approaches to optimize and simplify chronic hepatitis C (CHC) treatment, and patient monitoring and follow up.

2. To generate data on CHC treatment with G/P and associated clinical and economic outcomes of CHC treatment in populations of interest:
   a. Including but not limited to people who inject drugs (PWID), men that have sex with men (MSM), other active drug users, subjects in prisons, or ethnic or racial minorities as applicable for specific geography.

3. Impact of HCV treatment on extra-hepatic manifestations of HCV infection.

4. To explore novel approaches and technologies to optimize the HCV care cascade
   a. Screening and diagnosis:
      i. Reducing barriers to screening and diagnosis of populations thought to be at high risk of unknown HCV infection.
      ii. Approaches to HCV micro-elimination addressing a specific population either by geographic location or patient characteristics (e.g. renal care centers, prisons, specific rehabilitation centers, needle exchanges, opiate-substitution therapy and other addiction centers, hemophiliac cohorts and population from a region, city or country).
   b. Linkage to care:
      i. Improve access to care for newly diagnosed HCV patients.
   c. Treatment initiation and follow up:
      i. Use of innovative approaches including wearable technologies to help better understand the full benefits of achieving a cure, e.g. impact on lifestyle, physical activity, EHM, emotional well-being, quality of life, work productivity, societal economic benefit.

5. Support research that characterizes the population of HCV-infected individuals residing in various regions of the world which have limited or no epidemiological data with respect to:
   a. Included but not limited to range of comorbid conditions seen among HCV-infected patient population and the commonly used medications.
Immunology-Biotherapeutics - Compound: Adalimumab (HUMIRA)

- **Non-Medical Switch:** Non-clinical (e.g., analytical or pre-clinical), clinical, or patient-reported outcomes studies exploring the potential impact of switching among biologics, in particular between biosimilars and originator biologics, including treatment patterns (e.g., multiple switches, switches between biosimilars of the same originator product, and switching back to prior biologic).

- **Health Economic Outcomes:** Observational studies covering Health Services Research to determine impact of biologics on measures of health resource utilization, regardless of the product being an originator or biosimilar. Some examples include economic implications of non-medical switch, impact of mandated non-medical switch policies on patient outcomes, and societal impact of introduction of biosimilars.

- **Benefit/Risk:** Prospective and/or retrospective post-approval, observational studies, including registries, assessing real-world benefit:risk profile of biologics, regardless of the product being an originator or biosimilar.

- **Immunogenicity:** Non-clinical (e.g., analytical or pre-clinical), clinical, or observational studies assessing immunogenic profile of biologics, regardless of the product being an originator or biosimilar.

- **Safety Populations Identification:** Non-clinical, clinical, or observational studies exploring differences in patient susceptibility to immunogenic and/or other adverse events (including lack/loss of efficacy) among autoimmune inflammatory diseases or in special patient sub-populations, with focus on disease state rather than on any individual drug.

- **Patient Support Program:** Observational or interventional studies evaluating the impact of patient support programs on clinical and health outcomes (e.g., healthcare provider/clinician and patient-reported outcomes).

- **Pharmacovigilance:** Observational studies covering both pharmacy and medical channels tracking originator and biosimilar brands to assess traceability and attribution of adverse events.
Immunology-Dermatology - Compound: Adalimumab (HUMIRA)

**Dermatology/ Psoriatic Disease**

1. The role of TNF and/or IL-23 in the pathogenesis of psoriasis and/or psoriatic arthritis, and their associated comorbidities.

2. Non clinical, ex-vivo, or in-vitro research of TNF and/or IL-23 pathways in psoriasis and psoriatic arthritis.

3. Epidemiology of undiagnosed psoriasis and psoriatic arthritis, associated comorbidities and markers for early detection of psoriasis and psoriatic arthritis.

4. Markers, instruments and diagnostic tools to assess diseases progression, comorbidities, and optimization of response to HUMIRA.

5. Impact of early systemic treatment, treatment optimization, treatment goals and initiatives to advance quality of care in managing psoriasis.

6. Real World Evidence of long term use of HUMIRA and other biologics: clinical impact, patient reported outcomes, health economic and healthcare resource utilization associated with psoriatic diseases and comorbidities.

7. Understanding treatment compliance, persistence, adherence and clinical relevance of patient support programs for psoriatic diseases.

**Dermatology/ Hidradenitis Suppurativa (HS)**

1. Etiology, epidemiology, pathophysiology, classification and comorbidities associated with hidradenitis suppurativa.


3. Humanistic and economic burden of underdiagnosed/undertreated hidradenitis suppurativa, and the benefits of timely treatment with HUMIRA on disease progression.

4. Real World Evidence of HUMIRA: clinical impact, patient reported outcomes, health economic and healthcare resource utilization associated with hidradenitis suppurativa.

5. Integrated medical and surgical management of hidradenitis suppurativa and the impact on outcomes.

6. Understanding treatment compliance, persistence, adherence and clinical relevance of support programs for hidradenitis suppurativa.

**Dermatology/ Atopic Dermatitis**

1. Epidemiology, natural history, management, comorbidities, and humanistic/economic burden of atopic dermatitis.

2. Pathophysiology of atopic dermatitis including JAK1 signaling and novel therapeutic targets.

3. Tools and markers that aid diagnosis, classification, and evaluation of atopic dermatitis.
Immunology-Gastroenterology - Compound: Adalimumab (HUMIRA) and Upadacitinib

Clinical interventional studies with upadacitinib & risankizumab are not supported.

For preclinical studies utilizing upadacitinib, only *ex-vivo*, or *in-vitro* studies will be considered. Studies utilizing risankizumab will not be accepted in 2018.

1. Predictive and prognostic factors of disease severity and response to therapy in IBD (adult and pediatric)
2. Optimal monitoring of symptoms and inflammation in IBD (adult and pediatric)
3. Exploration of drug mechanisms and targeted therapy approaches in IBD
4. Broadening the knowledge about the role of early and sustained control of inflammation to prevent disease progression in IBD
5. Approaches to optimizing HUMIRA and/or concomitant therapies to improve outcomes in IBD (adult and pediatric) disease management. *Retrospective analyses only*
6. Real world effectiveness data for HUMIRA in UC and Pediatric CD. *Retrospective analyses only*
7. Global burden of IBD (total cost of illness, quality of life, co-morbidities) and total benefits of disease control with HUMIRA
8. Epidemiology of IBD in Latin America and China
9. Microbiome: role as a predictor of Loss of Response [LOR] and flare; changes in response to biologic treatment of IBD
10. Evaluation of novel and/or exploratory biomarkers in IBD
11. Exploration of mechanisms of disease (disease state and novel therapeutic targets in IBD)
12. Broadening the knowledge of IL23 and JAK 1 pathways in IBD.
Immunology-Rheumatology - Compound: Adalimumab (HUMIRA) and Upadacitinib

All the listed strategic priorities primarily apply to rheumatoid arthritis (RA), juvenile idiopathic arthritis (JIA), psoriatic arthritis (PsA) and axial spondyloarthritis (axial SpA).

Clinical interventional studies with upadacitinib & risankizumab are not supported.

For preclinical studies utilizing upadacitinib, only ex-vivo, or in vitro studies will be considered. Studies utilizing risankizumab will not be accepted in 2018.

1. New insights into disease course and pathology, burden of disease, including extra-articular manifestations, comorbidities and health economic outcomes, and impact of HUMIRA/anti-TNF therapy.

2. New insights into disease course, including structural progression and predictors thereof; disease modifying effects of HUMIRA/anti-TNF.

3. New approaches to and benefits of referral strategies, early diagnosis and/or early treatment, treatment targets, tight disease control and detection or monitoring tools, including imaging and biomarkers.

4. Further understandings of unmet needs and burden of disease in the treatment of RA including:
   1. Unmet need in patients treated with monotherapy
   2. Unmet needs in bDMARD-IR patients
   3. Humanistic burden of disease including pain, fatigue, morning stiffness and socio-economic impact.

5. Outcomes/effects of treatment strategies or treatment combinations enhancing understanding of HUMIRA and other systemic antirheumatic compounds, including prediction of flare and retreatment.

6. Impact of programs, instruments and tools aiming to improve patient care, persistence and adherence on clinical, patient report and health economic outcomes.

7. Evaluation of novel and/or exploratory biomarkers.

8. Exploration of mechanisms of disease (disease state and novel therapeutic targets).

9. Broadening the knowledge of IL-23 (PsA) and JAK 1 pathways (all indications).
Immunology-Ophthalmology (Uveitis) - Compound: Adalimumab (HUMIRA)

1. Burden of disease in non-infectious uveitis (includes comorbidities and disease related health economic impact and PRO/QoL outcomes)

2. Monitoring uveitis against 'treatment goals' - including measures of disease activity, prediction of treatment response impact of current therapy on long term outcomes, safety, comorbidities, and medical costs. Data on the prevalence and treatment patterns across different types of uveitis (including underlying IMID)

3. Treatment strategies for non-infectious intermediate, posterior or panuveitis including optimal timing for treatment escalation and timing for transitioning treatment to systemic immunosuppressants

4. Models of shared decision-making, communication among the multi-disciplinary team in the management of the uveitis patient.

5. Under-recognition of pediatric uveitis, consequences of disease, and best practices for transition to adult care
Neuroscience - Parkinson’s Disease – Compound: Duopa/Duodopa

1. Further understand the characteristics of advanced Parkinson’s disease (APD) patients and identify appropriate timing of treatment for optimal clinical outcomes

2. Define appropriate patient selection and appropriate proactive PD medication management to achieve monotherapy with levodopa/carbidopa intestinal gel (LCIG)

3. Evaluate safety/efficacy in subpopulations of APD patients including, but not limited to: patients with impulse control disorders (ICDs), freezing of gait, axial symptoms in whom oral treatments no longer offer benefits

4. Further evaluate LCIG and levodopa/dopaminergic response including, but not limited to:
   a. Bridging continuous drug delivery (CDD) to continuous dopaminergic stimulation (CDS)
   b. Modification of disease course
   c. Further understanding the characteristics of LCIG patient population and responders, clinical assessment tools, and key clinical and/or health economic outcomes, including but not limited to: i. Patient and caregiver-related outcomes; ii. Activities of daily living (ADL); iii. Quality of life (QoL); iv. Dyskinesia; v. NMS (non-motor symptoms).
   d. Safety and efficacy of outpatient titration, monotherapy, use for a duration of > 16 hours/day or > 1 cassette/day.
   e. Understanding the use of LCIG in combination with other “device-aided therapies” (e.g., those with uncontrolled tremor).

5. LCIG and safety observations in clinical practice including, but not limited to: procedure-related events

6. Evaluate safety and efficacy of LCIG in APD patients on multiple domains, such as motor and non-motor symptoms, ADL, and QoL by using wearable devices, biosensors or additional available technologies such as telemedicine.

7. Understanding LCIG’s role in the treatment paradigm in APD, including, but not limited to:
   a. Comparative efficacy/safety/functional outcomes to current and future treatments
   b. With adjunctive treatments
   c. Safety/efficacy in non-responders to other PD therapies

8. Additional focus areas:
   a. Understanding the impact of multi-disciplinary care models in APD and Interventions or systems to reduce caregiver burden
   b. Enhancement in diagnosis and ongoing evaluation of APD by utilizing patient assessment tools and/or technologies
   c. Identify the impact of microbiomes in the response to LCIG in patients with APD
   d. Evaluation of new device/pump/tubing technologies with the goal to enhance safety and efficacy of LCIG delivery
   e. Acquisition of data regarding LCIG device and its use, including, but not restricted to, pump, tubing, titration, and human factor.

Epidemiology of Movement Disorders, including identification of patient groups and subgroups.
Depatux-M (ABT-414) is an antibody-drug conjugate (ADC) that selectively targets cells with EGFR amplification. Depatux-M (ABT-414) is being developed in glioblastoma. Based on their early stage of development, consideration will only be given to limited preclinical and clinical study applications in 2018.

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

- Optimization of Depatux-M therapy across lines of treatment
- Novel therapeutic combinations
- Novel preclinical models (in vitro and in vivo)
- Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)
- Novel endpoints/additional endpoints
- Other EGFR-amplified tumor types

Things to consider for the investigator:

- 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals.
- Data for justification of study rationale preferred.
Oncology - Compound: Navitoclax (ABT-263)

Navitoclax (ABT-263) is being developed in multiple hematological and solid tumor disease states. Based on their early stage of development, consideration will only be given to limited preclinical and clinical study applications in 2018.

Priority will be given to applications proposing to investigate the following areas:
- Novel therapeutic combinations
- Novel clinical indications
- Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)
- Novel endpoints

Things to consider for the investigator:
- 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals.
- Proper inclusion/exclusion criteria for clinical proposals, along with adequate safety precautions, monitoring and reporting
Oncology - Compound: Rovalpituzumab Tesirine (Rova-T)

Rovalpituzumab tesirine (Rova-T) is an antibody-drug complex (ADC) targeted to DLL3 which is expressed on the surface of neuroendocrine tumors including SCLC. Consideration will be given to both preclinical and clinical applications. However, based on clinical stage of development where the risk, benefit and safety profiles are still being assessed in different disease states, limited preclinical and clinical proposals will be supported in 2018.

Priority will be given to applications proposing to investigate the following areas:
- Therapeutic combinations including novel agents where there is scientific rationale
- Increasing the scientific body of evidence evaluating DLL3 as a biomarker
- Novel clinical indications where there is scientific rationale
- Novel Endpoints

Things to consider for the investigator:
• 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals
Oncology - Compound: Veliparib (ABT-888)

Veliparib is a PARP inhibitor with scientific rationale for therapeutic impact across a broad variety of tumor types. Consideration will be given to both preclinical and clinical applications in 2018. Based on the early clinical stage of development where the risk, benefit and safety profiles are still being assessed in different disease states, there will be limited clinical proposals considered.

Priority will be given to applications proposing to investigate the following areas:
- Novel therapeutic combinations
- Novel clinical indications where there is scientific rationale
- Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)

Things to consider for the investigator:
- 2018 limited ability to support proposals that require monetary funding outside of drug only proposals.
Oncology - Compound: Venetoclax (ABT-199)

Venetoclax is a BCL2 inhibitor with scientific rationale for evaluation across a broad variety of Hematologic malignancies and solid tumors. *Consideration will be given to both preclinical and clinical applications in 2018.*

Priority will be given to applications proposing to investigate the following areas:
— Novel therapeutic combinations
— Novel clinical indications where there is scientific rationale
— Evaluation of novel and/or exploratory predictive models or biomarkers
— Evaluation of novel and additional clinical endpoints

**Notable considerations for the investigator:**
• Limited funds may be available to support proposals
Oncology - Compound: Telisotuzumab-Vedotin (ABBV-399)

Telisotuzumab -vedotin (ABBV-399) is a c-MET antibody-drug conjugate with a scientific rationale across a broad variety of malignancies. Based on their early stage of development, consideration will only be given to limited preclinical and clinical study applications in 2018.

Priority will be given to applications proposing to investigate the following areas:
  • Novel therapeutic combinations
  • Novel clinical indications where there is scientific rationale
  • Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)

Things to consider for the investigator:
  • 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals
Oncology - Compound: ABBV-621

ABBV-621 is a fusion protein TRAIL agonist with a scientific rationale across a broad variety of malignancies. Based on their early stage of development, consideration will only be given to limited preclinical and clinical study applications in 2018.

Priority will be given to applications proposing to investigate the following areas:
• Novel therapeutic combinations
• Novel clinical indications where there is scientific rationale
• Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)

Things to consider for the investigator:
• 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals.
**Oncology - Compound: ABT-165**

ABT-165 is a VEGF and DLL4 targeted dual variable domain antibody with a scientific rationale across a broad variety of malignancies. Based on their early stage of development, *consideration will only be given to limited preclinical and clinical study applications in 2018*.

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

- Novel therapeutic combinations
- Novel clinical indications where there is scientific rationale
- Evaluation of novel and/or exploratory biomarkers (disease state and therapeutic targets)

**Things to consider for the investigator:**

- 2018 limited ability to support proposals that require monetary funding outside of drug-only proposals.
Pancreatology - Compound: Creon

1. Efficacy of CREON (e.g. CREON dose-response, effects in nutrition, body composition, weight, physical activity, QoL) in patients with exocrine pancreatic insufficiency (EPI) due to the following:
   a. Chronic Pancreatitis
   b. Pancreatectomy
   c. Cystic Fibrosis
2. Impact of CFTR modulators on EPI management in CF patients
3. Disease burden / Prevalence of EPI in
   a. Diabetes (Type 1, 2, and 3c)
   b. IBS
   c. Celiac Disease
   d. IBD
   e. Special populations (lysosomal storage diseases/cystinosis, geriatric patients with malnutrition, etc.)
4. New diagnostic methodologies/biomarkers for EPI.
Women’s Health - Benign Gynecologic Diseases

Endometriosis Areas of Interest

1. Broadening the knowledge about the disease course and natural history of endometriosis
   - Burden of disease
   - Cumulative Impact
     - On Pain
     - On Infertility
     - On quality of life (QOL) domains
   - Co-morbidities
   - Health economic implications
2. Improving approaches to making a non-surgical diagnosis of endometriosis
3. Optimal monitoring of response to endometriosis treatments & algorithms of care
   - Exploring elagolix dosing strategies
4. Evaluation of the economic benefits of Elagolix treatment
5. Differentiation of GnRH analog MOA and clinical implications (including appropriate in vivo and in vitro non-human models)
6. Novel clinical applications of Elagolix with a focus on Women’s Health
Neonatology – Compound: Synagis (JAPAN ONLY)

1. Risk and associated morbidity and mortality of severe RSV infection among infants and children with underlying medical conditions or chronic diseases.