# *in*Thought Capabilities

September 2020

## Rare Genetic Disease and Rare Disease

inThought®

# What Makes *in*Thought Unique?

## business intelligence

*A* multidisciplinary team of 16 subject matter experts with deep therapeutic area expertise that provides business intelligence support to pharmaceutical, biotech, and financial companies.

Our decision support uses a variety of data points, both primary & secondary, combined with analysis & insight. This methodology allows clients to triangulate actionable intelligence and adjust strategy.

## analysis & insight

## business & science

We understand the business and science. *in*Thought principals are MDs, PhDs, and MBAs with clinical, research, regulatory, and Wall Street experience.

Our proprietary *in*Vision platform allows clients to have all critical intelligence in one place, customized for their particular key questions and topics, and accessible on demand.

## proprietary platform

# *in*Thought Competitive Intelligence Expertise

*inThought is able to support a diverse body of client work.*

## Continuous Monitoring

- **Email alerts** with analysis
- **Competitive Monitoring Reports** (pipeline view, launch timelines, trial design, etc., updated periodically)
- **Landscape Assessments**
- **Social Media Monitoring** (drugs, TAs, KOLs, etc.)
- KIT and KIQ Tracking
- Analyst support
- Ongoing primary interviews with competitors, physicians, & KOLs

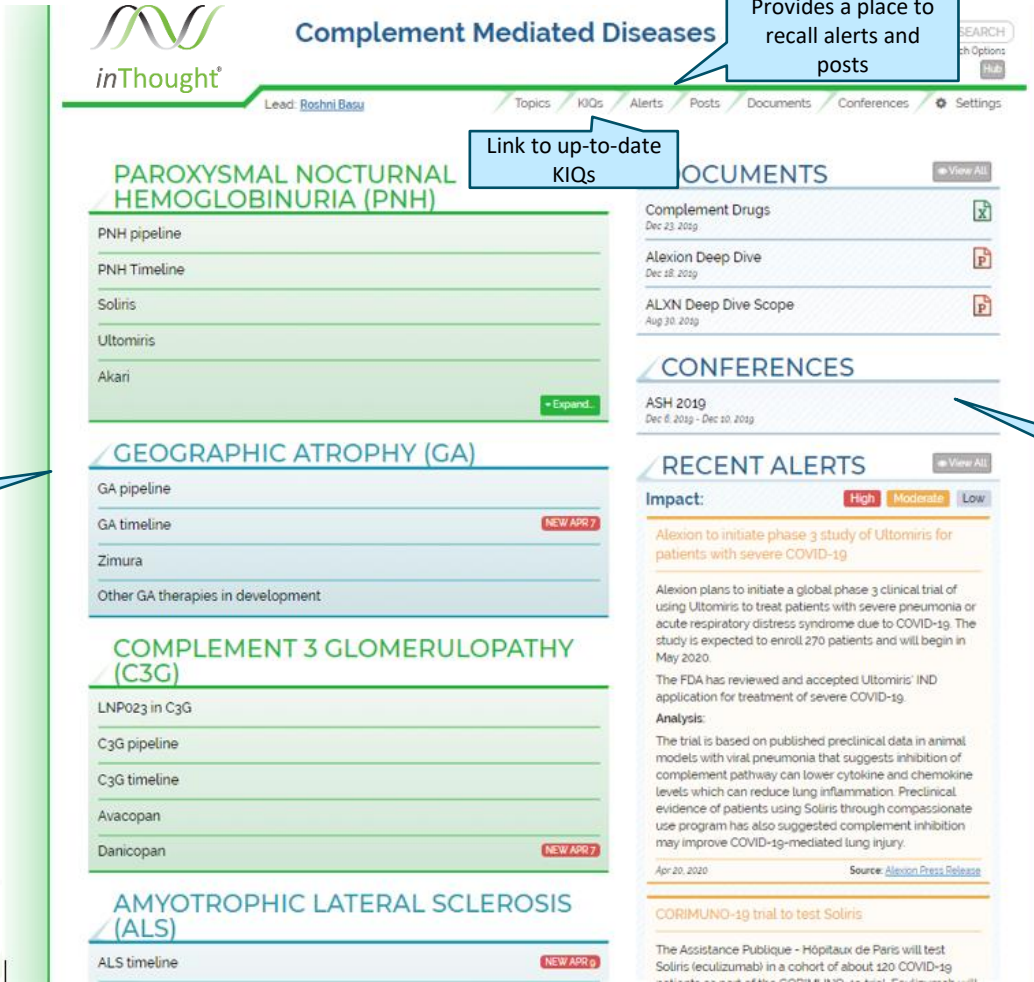
## Conference Coverage

- Focus on presenter and attendee feedback
- KOL engagement
- Booth messaging
- Future messaging
- Social Media Monitoring (conference name, TAs, drugs, etc.)
- Address KIQs and KITs

## Ad Hoc Projects

- Trial design analyses
- Primary research
- KOL interviews and social media influencer analysis
- Revenue forecasts
- Probability of approval
- Deep Dive assessments of companies, drugs, & MOAs
- Target Product Profiles
- Licensing opportunities
- Strategy workshops
- BD due diligence

# *in*Vision Dashboard Example




Provides a place to recall alerts and posts

Link to up-to-date KIQs

Topic folders organized by theme

Curated documents for the site

Links to conference data

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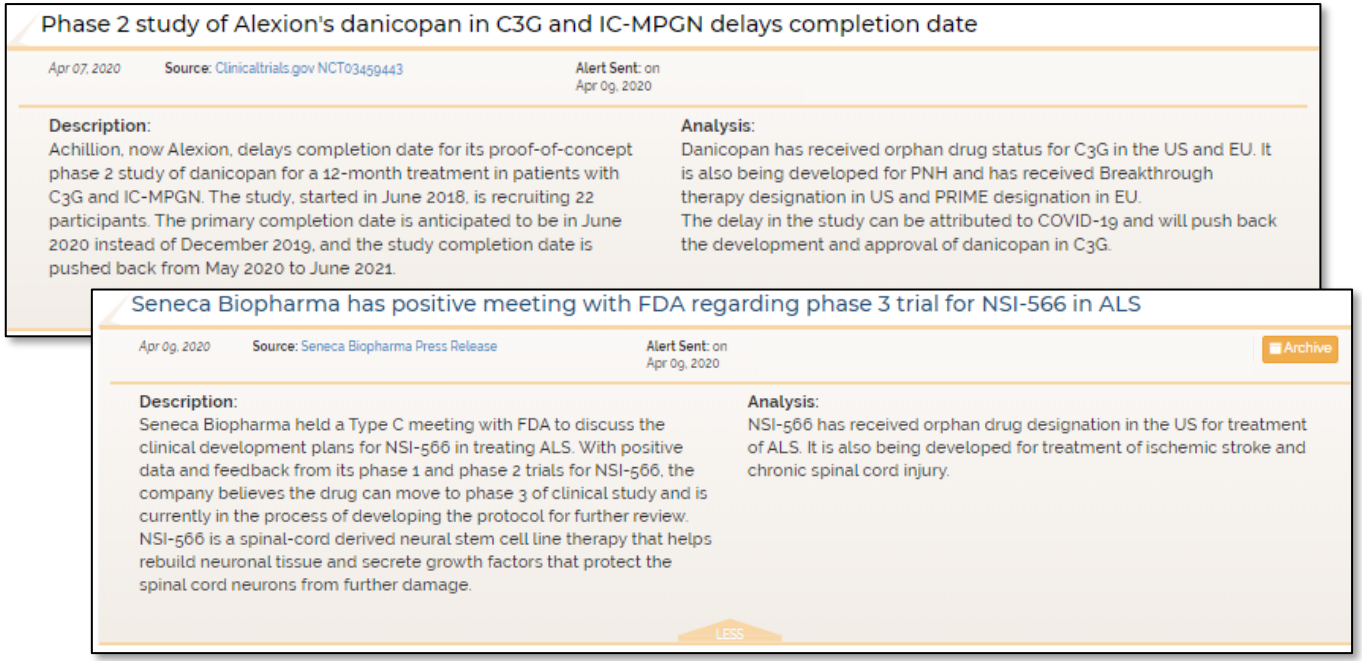
# Continuous Monitoring Examples (1)
## *Email Alerts*

*inVision connects CI teams to timely alerts and strategic implications based on therapeutic areas, clinical trials, and MOAs of interest.*



Phase 2 study of Alexion's danicopan in C3G and IC-MPGN delays completion date

Apr 07, 2020 **Source:** Clinicaltrials.gov NCT03459443 **Alert Sent:** on Apr 09, 2020

**Description:**
Achillion, now Alexion, delays completion date for its proof-of-concept phase 2 study of danicopan for a 12-month treatment in patients with C3G and IC-MPGN. The study, started in June 2018, is recruiting 22 participants. The primary completion date is anticipated to be in June 2020 instead of December 2019, and the study completion date is pushed back from May 2020 to June 2021.

**Analysis:**
Danicopan has received orphan drug status for C3G in the US and EU. It is also being developed for PNH and has received Breakthrough therapy designation in US and PRIME designation in EU.
The delay in the study can be attributed to COVID-19 and will push back the development and approval of danicopan in C3G.

Seneca Biopharma has positive meeting with FDA regarding phase 3 trial for NSI-566 in ALS

Apr 09, 2020 **Source:** Seneca Biopharma Press Release **Alert Sent:** on Apr 09, 2020 Archive

**Description:**
Seneca Biopharma held a Type C meeting with FDA to discuss the clinical development plans for NSI-566 in treating ALS. With positive data and feedback from its phase 1 and phase 2 trials for NSI-566, the company believes the drug can move to phase 3 of clinical study and is currently in the process of developing the protocol for further review.
NSI-566 is a spinal-cord derived neural stem cell line therapy that helps rebuild neuronal tissue and secrete growth factors that protect the spinal cord neurons from further damage.

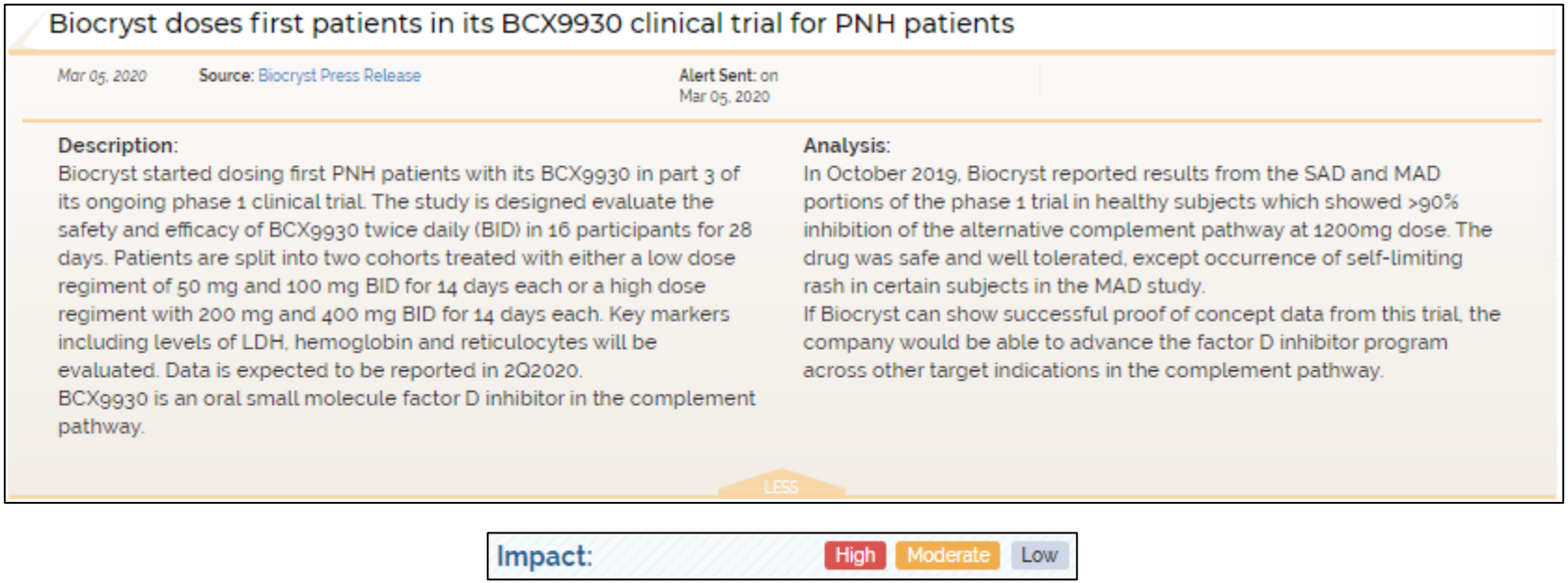
**Analysis:**
NSI-566 has received orphan drug designation in the US for treatment of ALS. It is also being developed for treatment of ischemic stroke and chronic spinal cord injury.

LESS

**Impact:** High Moderate Low

- inThought analysts continually monitor ***corporate press releases***, ***investor presentations***, ***conference abstracts***, and ***clinical trial records*** to provide timely analysis of relevant events
- Analysis is customized and includes implications that are specific to the client's portfolio
- These alerts are prioritized on inVision as High, Medium, and Low priorities and emailed to client's CI teams
- inVision allows CI teams to keep a pulse on key therapeutic areas, clinical trials, and MOAs

# News Alert Example



## Biocryst doses first patients in its BCX9930 clinical trial for PNH patients

*Mar 05, 2020* **Source:** Biocryst Press Release **Alert Sent:** on Mar 05, 2020

**Description:**
Biocryst started dosing first PNH patients with its BCX9930 in part 3 of its ongoing phase 1 clinical trial. The study is designed evaluate the safety and efficacy of BCX9930 twice daily (BID) in 16 participants for 28 days. Patients are split into two cohorts treated with either a low dose regiment of 50 mg and 100 mg BID for 14 days each or a high dose regiment with 200 mg and 400 mg BID for 14 days each. Key markers including levels of LDH, hemoglobin and reticulocytes will be evaluated. Data is expected to be reported in 2Q2020.
BCX9930 is an oral small molecule factor D inhibitor in the complement pathway.

**Analysis:**
In October 2019, Biocryst reported results from the SAD and MAD portions of the phase 1 trial in healthy subjects which showed >90% inhibition of the alternative complement pathway at 1200mg dose. The drug was safe and well tolerated, except occurrence of self-limiting rash in certain subjects in the MAD study.
If Biocryst can show successful proof of concept data from this trial, the company would be able to advance the factor D inhibitor program across other target indications in the complement pathway.

LESS

**Impact:** High Moderate Low

# Continuous Monitoring Examples (2)
## *Email Alerts*

*inVision connects CI teams to timely alerts and strategic implications based on therapeutic areas, clinical trials, and MOAs of interest.*

### Clinical Trial Monitoring

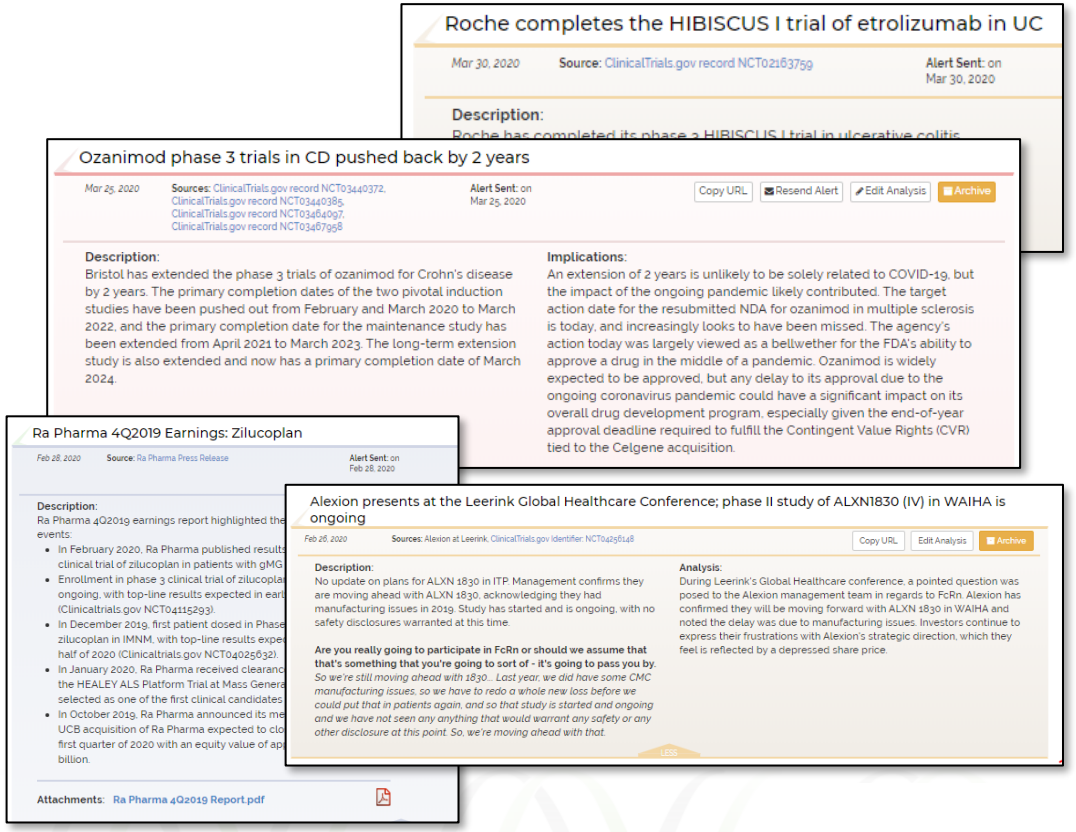
- Trial results (e.g., press release)
- Trial delays
- Trial completions
- New trials
- Endpoint updates
- Trial size expansions

### Investor Presentations

- Management commentary and strategy
- Q&A capture

### Earnings Coverage

- Revenue tracking
- Industry trends
- Management commentary and strategy
- Q&A capture



# Competitive Intelligence (CI) Landscape

## Sample Work

# Continuous Monitoring Overview


| Topic | Slide |
|---|---|
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# Catalyst Tracker
## *Sickle Cell Disease*

| Event | Expected Timing |
|---|---|
| NHLBI SCD Advisory Committee meeting | May 31, 2019 |
| Results from full HOPE **voxelotor** study to be presented at EHA | June 2019 |
| **Crizanlizumab** regulatory filing | 1H19 |
| First SCD patient expected to be infused with **CTX001** | mid-2019 |
| **Voxelotor** filing | 4Q19 |
| Initiation of confirmatory **voxelotor** TCD study | 2H19 |
| Initiation of phase 3 **LentiGlobin** HBG-210 study | by end 2019 |
| **Inclacumab** IND submission | 2021 |
| **LentiGlobin** filing | 2022 |

Note: As of May 31, 2019

# Disease Overview: Sickle Cell Disease



## The burden of SCD is significant

- Estimated U.S. prevalence: 100,000 individuals
- Life expectancy (U.S.): 45-58 years
- Annual U.S. healthcare costs: over $1B

## Current therapies are limited

**Prevention of complications**

- Hydroxyurea
- L-glutamine

**Treatment of complications**

- Pain management
- Antibiotics

**Curative**

- Hematopoietic stem cell transplantation

## Gene editing/replacement and other drug development and regulatory advances have renewed interest in SCD

- Potential for at least 3 new SCD approvals in the next 3 years (crizanlizumab, LentiGlobin, voxelotor)
- FDA's recent willingness to accept hemoglobin levels as a surrogate endpoint is a watershed moment for the field

## SCD may pose unique challenges for marketers of new agents

- Access to higher priced therapies may be an issue for some SCD patients, especially in developing regions
- SCD patients are perceived to be skeptical of the medical establishment and poorly compliant; these observations could be biased or related to the relative lack of acceptable therapies at present

# Pipeline Overview: Sickle Cell Disease

## *Preclinical Pipeline*

Pipeline Landscape

| | | | | |
|---|---|---|---|---|
| **Mitapivat;** Agios *PKR activator* | **ARQ 092**; PO ArQule, Univ or Illinois *Pan-AKT inhibitor* | **EdX-17**; parenteral EpimedX *Plant growth factor* | **DRX-194** DeuteRx *Deuterium stabilized single enantiomer* | **Undisclosed**; IV Maxcyte/NIAID *Non-viral CRISPR mediated gene therapy* |
| **AIC-6020** PHD Biosciences *Antioxidant (unspecified)* | **HBI 002**; PO Hillhurst Bio *Heme oxygenase modulator* | **MEDI 6012 (ACP501)**; IV AstraZeneca *Recombinant human LCAT* | **Anti-inflammatory enzyme inhibitors;** PO PHD Biosciences | **No designation** Homology/Novartis *AAV based gene therapy* |
| **PNQ 103**; PO Impetis *Adenosine A2B receptor antagonist* | **NM-96**; PO PHD Biosciences *Antioxidant* | **Vacno** SynZyme Technologies *Caged NO labeled albumin* | **No designation**; PO Orphagen Pharmaceuticals *Orphan nuclear receptor antagonists* | **No designation** Fulcrum Therapeutics *Small molecule gamma globin activator* |
| | | | **No designation**; IV CSL Behring *Plasma derived haptoglobin and hemopexin* | **No designation**; IV Genethon/INSERM, Stanford U *Lentiviral gene therapy targeting HBB* |
| | | | **Keap1ASO** Ionis *Antioxidant genes inducer* | **No designation**; IV Editas *CRISPR/CAS9 targeting HBB* |
| | | | **Klf1ASO** Ionis *gamma-globin inducer through regulation of BCL11A* | **Sirolimus (rapamycin)** Rare Partners, Univ. of Ferrara *HbF inducer* |
| | | | **Undisclosed** Syros *Single gene modulator* | |

*Antioxidant competitor*

| | | | |
|---|---|---|---|
| Small Molecule | Protein | Polysaccharide | Cell Therapy |
| Monoclonal Ab | Gene Therapy | RNAi / nucleic acid | Undisclosed |

# Pipeline Overview: Sickle Cell Disease

## *Clinical Pipeline*

Pipeline Landscape

| PHASE I | PHASE I/II | PHASE II | PHASE III | MARKETED |
|---|---|---|---|---|
| **Farydak**(panobinostat); PO** Novartis *HDAC inhibitor* | **LentiGlobin**(BB305); IV Bluebird Bio *santi-sickling beta-globin gene* | **IMR-687**; PO Imara *PDE9A inhibitor* | **Brilinta (ticagrelor)**; PO AstraZeneca *$P2Y_{12}$ platelet inhibitor* | **Droxia** (hydroxyurea); PO Bristol-Myers Squibb *SC hemoglobin polymerization inhibitor* |
| **EPI-01**; PO EpiDestiny/Novo *cytidine deaminase inhibitor* | **CTX-001**; IV CRISPR Therapeutics/Vertex *CRISPR/Cas9 targeting BCL11A* | **Sanguinate**; IV Prolong *dual mode CO and $O_2$ delivery therapeutic (pegylated Hb)* | **rivipansel** (GMI-1070); IV Pfizer/GlycoMimetics *pan-selectin inhibitor (E, L, P)* | **Siklos** (hydroxyurea); PO Addmedica; age: 2+ *SC hemoglobin polymerization inhibitor* |
| **PF-04447943***; PO Pfizer *PDE9 inhibitor* | **inclacumab**; IV Global Blood Therapeutics/Roche *pan-selectin inhibitor* | **Sevuparin**; IV Modus *cell adhesion molecule inhibitor* | **voxelotor** (GBT440); PO Global Blood Therapeutics *sickle Hb modulator* | **Endari** (L-glutamine); PO Emmaus *antioxidants, protein synthesis modulators* |
| **SCD-101**; PO Invenux *botanical sickling inhibitor* | **BIVV-003,** IV Bioverativ (Shire)/Sangamo *ZFN editing targeting BCL11A* | **Ilaris** (canakinumab); IV Novartis *IL-1b mAb* | **crizanlizumab** (SELG1/SEG101); IV Novartis *P-selectin inhibitor* | |
| | | **IW-1701** (olinciguat); PO Cyclerion (Ironwood) guanylate cyclase agonist | | |

Small Molecule

Monoclonal Ab

Protein

Gene Therapy

Polysaccharide

*acute treatment only*



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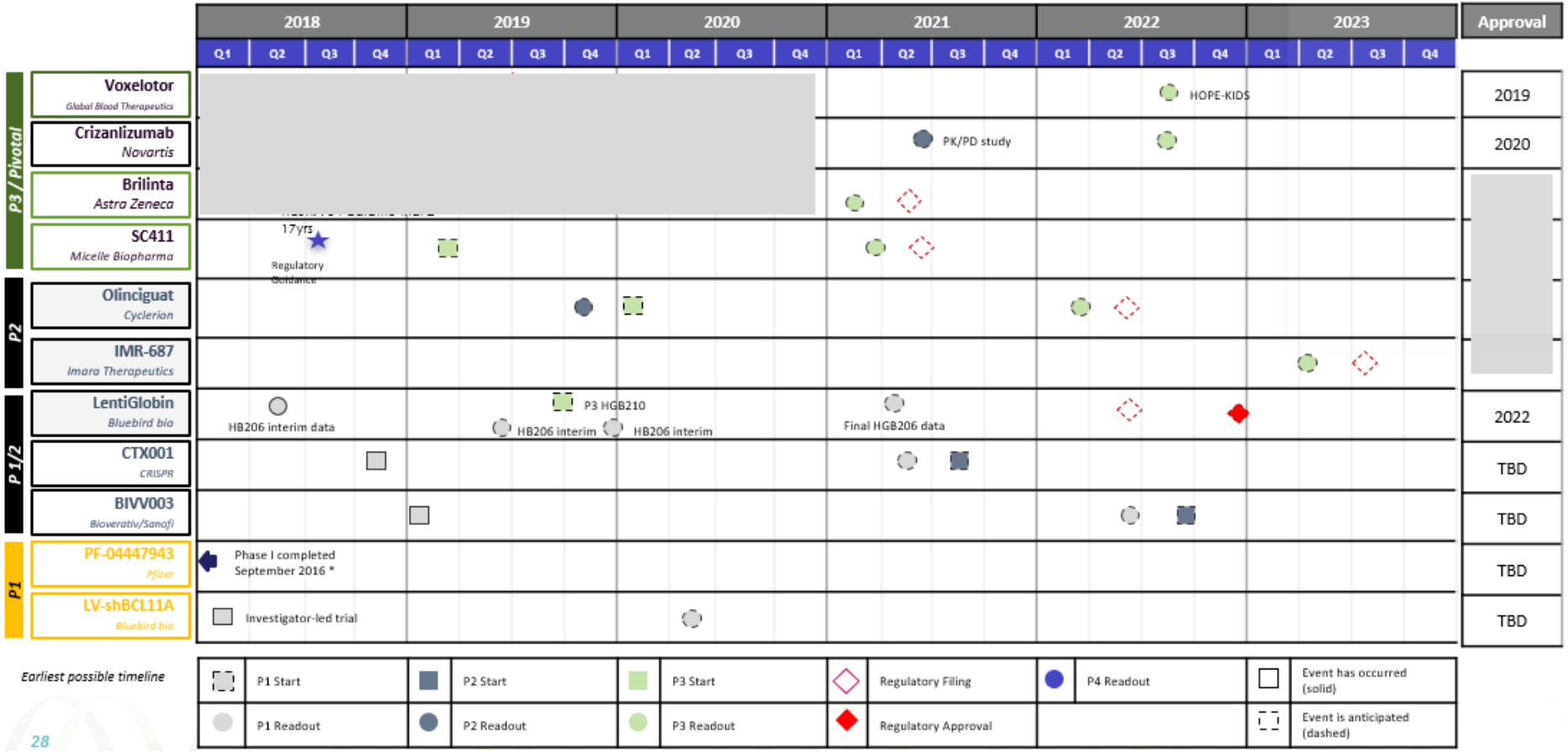
# Development Timelines: Sickle Cell Disease






*Earliest possible timeline*

* Program is listed on Pfizer's latest pipeline overview, dated April 30, 2019, although development appears to have stalled

# Clinical Trial Overview

# Sample Work

# Clinical Trial Overview: Sickle Cell Disease

## *Voxelotor (GBT440)*

Clinical Trial Overview

| Trial ID | Phase | Patient Population | Trial Length | Arms | Key Trial Endpoints |
|---|---|---|---|---|---|
| **HOPE**<br>NCT03036813<br>*fully enrolled* | 3 | **Inclusion**<br>• Age 12-65<br>• At least one episode of VOC in the last 12 months<br>• Hb ≥ 5.5. and ≤ 10.5 g/dL<br>**Exclusion**<br>• >10 VOCs requiring clinic visit<br>• Regular RBC transfusion | Start Date: Dec. 2016<br>**Primary completion**: Oct. 2019<br><br>Estimated Length: 2 years, 10 months | • GBT440 (Dose 1)<br>• GBT440 (Dose 2)<br>• Placebo<br><br>N=300 | **Primary**<br>• Proportion with increase of Hb > 1g/dL (baseline to 24W)<br>**Secondary**<br>• Change in hemolysis measures (24W)<br>• Annual rate of VOCs (72W) |
| NCT02850406<br>*recruiting* | 2 | **Inclusion**<br>• Age 4-17 (child)<br>• HbSS or HbS β0 thalassemia<br>• Hb ≤ 10.5 g/dL (parts B & C)<br>**Exclusion**<br>• VOC or ACS (within 14 days of signing consent)<br>• Requires chronic transfusion<br>• Transfusion in past 30 days | Start Date: May 2016<br>**Primary completion**: May 2022<br><br>Estimated Length: 6 years | • Part A – GBT440, PO, 1 day (single dose)<br>• Part B – GBT440, PO, QD to 24W<br>• Part C – GBT440, PO, QD to 48W<br><br>N=125 | **Primary**<br>• PK (Part A)<br>• Change in hemoglobin (Part B, 24W)<br>• Change in cerebral blood flow via TAMM TCD velocity (Part C, 48W)<br>**Secondary**<br>• Clinical measures of hemolysis<br>• TEAEs<br>• PK |
| NCT02285088<br>SAD/MAD study<br>*completed* | 1 | **Inclusion**<br>• 18-60 (adult) with SCD<br>• Healthy volunteers included<br>**Exclusion**<br>• Alcohol consumption restrictions<br>• SCD and hemoglobin level <6 g/dL or >10.4 g/dL | Start Date: Dec. 2014<br>**Primary completion**: Mar. 2017<br><br>Estimated Length: 2 years, 3 months | • GBT440; PO,QD<br>• Placebo<br><br>N=133 | **Primary**<br>• AEs<br>**Secondary**<br>• Blood and plasma concentration<br>• % of hemoglobin occupied or modified by GBT440 |

# Clinical Trial Endpoint Comparison

## *Sickle Cell Disease*

Clinical Trial Overview

*The majority of Phase 3 SCD endpoints focus on VOCs; only voxelotor is using change in hemoglobin levels as a primary endpoint in Phase 3. LentiGlobin is also assessing Hb levels in a Phase 1/2 trial, with plans to do the same in Phase 3.*

| | | | | | | Key Endpoints | | | |
|---|---|---|---|---|---|---|---|---|---|
| | Drug | Trial | Age | Size | Estimated Length | Change in Hb | Days with SCD symptom exacerbation | Rate of VOCs requiring clinic visit | Rate/number of VOCs |
| Phase 3 | voxelotor | HOPE NCT03036813 | 12-65 | 400 | 2 years, 6 months | 1° | 2° | | |
| Phase 3 | crizanlizumab | STAND NCT03814746 | 12+ | 240 | 3 years, 1 month | | | 1° | 2° |
| Phase 3 | Brilinta | HESTIA3 NCT03615924 | 2-17 | 200 | 2 years, 1 month | | | 2° | 1° |
| Phase 3 | SC411 | ASCENT NCT02604368 | 5-17 | 210 | 1 years, 9 month | | | 2° | 1° |
| Phase 1/2 | LentiGlobin | HGB-206 NCT02140554 | 12-50 | 50 | 6 years, 5 months | 1° | | | 2° |

**Other Endpoints of Interest**

- Opioid and non-opioid use
- Time to first crisis
- Number of ACSs (Acute Chest Syndrome)
- Overall rates of hospitalization
- Number of days hospitalized

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# Primary Research

# Sample Work

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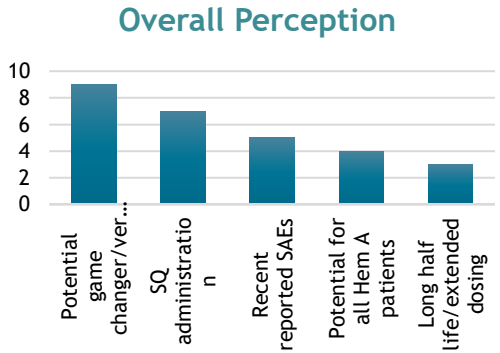
# Primary Research: Hemophilia

## *US/EU Physician Interviews (Perception of Emicizumab)*

*Potential in inhibitor patients, SQ administration, and the recent reported SAEs were top of mind with physicians (n=15) when asked about their perception of emicizumab.*

In response to an initial question about their overall perception of emicizumab:

- 9 of 15 physicians viewed the drug as a potential game changer or potentially very beneficial for patients with inhibitors
    - Only 4 physicians initially discussed emicizumab's potential for all hemophilia A patients
- Many physicians also highlighted SQ administration as well as the potential for extended dosing
    - Relative to extended dosing, most physicians and nurses discussed emicizumab as a once weekly drug
- 5 physicians mentioned the reported thrombotic SAEs up front, for some this was the first topic mentioned




DETAILED FINDINGS

QUOTES

- "Will be a fabulous drug for patients with inhibitors. Safety is a major issue, with current information will not use it in patients without inhibitors" – *German physician*
- "Efficacy reports are good. Sub Q. Patients who have been in trial like the drug. Recent AE report have shaken some of the enthusiasm about the drug" – *U.K. Physician*
- "Home run for inhibitor patients. For non-inhibitor patients, already have a safe and effective therapy. ACE 910 needs to as safe and effective. Sub Q very important advantage" – *U.S. Physician*
- "Will be a big splash when it comes out, but still lot of details to be ironed out that could make a difference on how much it's utilized." – *U.S. physician*

# Primary Research: Gastroenterology

## *KOL Interviews (n=5)*

| | KOL 1 | KOL 2 | KOL 3 | KOL 4 | KOL 5 |
|---|---|---|---|---|---|
| 8. What type of physician sees these patients? Who is the lead treater? | Many come straight to the gastro. This allows the XX to get more attention. Some might mention it to a PCP during a physical or have it noticed incident to something else | I think the majority see a primary care doc. Those seeing a gastroenterologist are going to be the severe ones. | It depends on insurance coverage. Some don't have a choice and are forced to see a PCP, while others have direct access. If XX is the primary complaint, then best to see a gastro. | I'm in private practice and because of insurance the types of patients I see are not going to the PCP for this | Definitely gastros see the more severe cases. Sometimes PCPs feel comfortable and some still refer to gastro practices. |
| ***Consensus*** | ***Severe XX patients are most likely to be treated by their gastroenterologists. Mild cases, the majority, are handled by a PCP.*** | | | | |
| | | | | | |
| 9. Is XX their primary complaint? | We see it as both a primary complaint and secondary issue. It is probably evenly distributed. | There are some, but mostly only the severe ones. The majority of folks are diagnosed incidentally to something else. | Often it is a secondary situation, especially for mild patients. If XX is the main feature you'd want to see gastro. | If it's effecting the lower quadrant I would say 80% of the time they are coming in for something else. 20% of patients come in just for XX. | Severe patients usually bring it up, however I think it's more common that patients don't since most are mild. |
| ***Consensus*** | ***Severe patients and some moderate will present with XX as their primary complaint. For mild cases, XX is commonly observed incident to other medical issues. KOLs suggest possibly a 20%/80% split for XX being a primary complaint vs. secondary complaint for patients.*** | | | | |

# Earnings Coverage
# Sample Work

# Earnings Coverage (1)
## *Executive Summary*

*Coverage of quarterly earnings calls provides key insights on management strategy, pipeline updates, and details from sell-side analyst Q&A.*

### Executive Summary: AbbVie 4Q19 Earnings

abbvie

**Topline Financials**
- ***FY2019:*** sales for AbbVie totaled $33.3 billion, up 2.7%
- ***4Q19***: sales for AbbVie totaled $8.7 billion, up 5.3%
- ***FY2019***: sales for Immunology were $15.2 billion, down (0.8%)
- **4Q19**: sales for Immunology were $5.17 billion, up 5.5%

**Product Performance**
- **Humira**: FY2019 sales were $19.2 billion, representing 0.5% growth. 4Q19 sales were $4.92 billion, down (2.9%).
  - 4Q19: U.S net revenues of $3.97B, up 9.8%; international net revenue of $948M, down (27%). Notable unfavorable impact of international biosimilar competition.
  - FY2019: U.S. sales up 8.6%
- **Skyrizi**: FY2019 sales were $355 million. 4Q19 sales were $216 million.
- **Rinvoq**: FY2019 sales were $33M. 4Q19 sales were $47M.

**Guidance**
- AbbVie expects to deliver standalone adjusted diluted EPS for the full-year 2020 of $9.61 to $9.71, representing growth of 8.1% at the midpoint.
- AbbVie expects standalone revenue growth approaching 8.0% on an operational basis
- AbbVie to provide combined guidance with Allergan after the close of the deal

**Pipeline**
- **Rinvoq**: 2Q20, PsA regulatory submission
- **Rinvoq**: 2H20, AS submission based on positive data presented at ACR 2019
- **Rinvoq**: mid-2020, phase 3 data in AD with submission plans for later in 2020
- **ABBV-3733**: TNF-steroid conjugate – early clinical efficacy data expected later this year (2020). Has the potential to serve as a platform across a wide range of indications for RA, IBD, and lupus. AbbVie is excited about this asset.
- **Skyrizi**: 2021, regulatory submissions for PsA and CD (phase 3 data anticipated in 2020)

**Q&A**
- Management states that Skyrizi and Rinvoq launches are ahead of schedule and therefore anticipated to outperform prior estimates.
- Management notes that ABBV-3733, a TNF-steroid conjugate, could deliver transformational results in its phase 2 proof-of-concept trial. This asset is deemed to have tremendous potential.

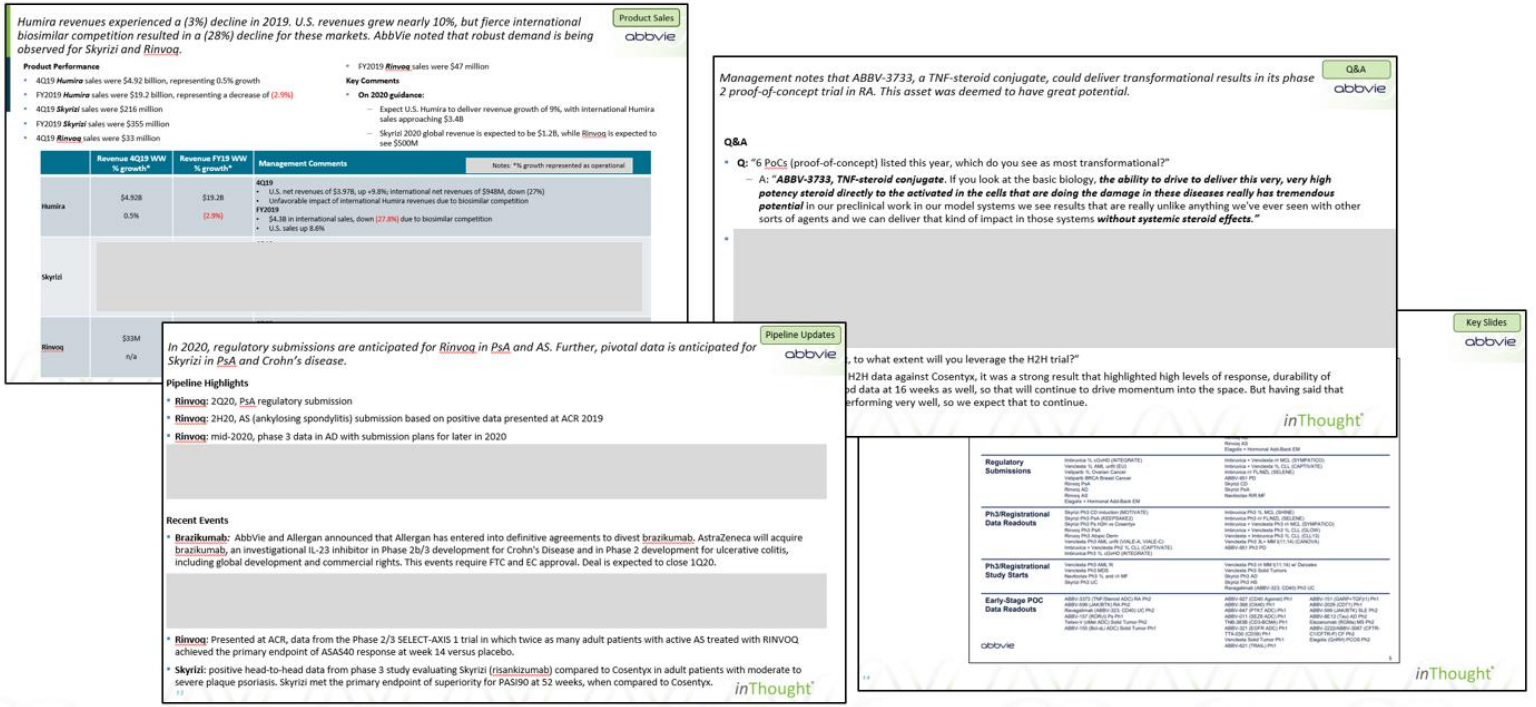
| WW Sales | 2017 actual ($M) | 2018 actual ($M) | 2019 actual ($M) | 2020 consensus ($M) | 2021 consensus ($M) |
|---|---|---|---|---|---|
| Humira | 18,427 | 19,936 | 19,169 | ↑ 19,209 (6) | ↑ 19,396 (6) |
| Skyrizi | --- | --- | 355 | ↑ 1,088 (6) | ↓ 1,830 (6) |
| Rinvoq | --- | --- | 47 | ↓ 393(5) | ↓ 885 (6) |

Consensus estimates compiled by Bloomberg, US$M (# analysts)

*Arrows signify the direction of change for estimates from the previous quarter to the current one.

# Earnings Coverage (2)
## *Additional Details*

*Comprehensive details are provided for revenue drivers, pipeline updates, key presentation slides, and relevant Q&A.*



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# Strategy Workshops

*in*Thought can lead competitive workshops. The exact focus and scope of these workshops will be determined by the client but could include focusing on competitive position in a specific TA, preparation for a new launch, or for a competitor launch.

| | |
|---|---|
| Workshop Design | *in*Thought will work with the client to develop the design of the workshop so that it is appropriate for the attendees and effective at developing insights for the potential market |
| Pre-read Materials | *in*Thought will prepare pre-read material for the workshop participants that will include clinical data, trial design, competitor strategy assessments, and other relevant data |
| Workshop Deck and Material | *in*Thought will develop, in consultation with the client, the workshop deck as well as any graphics or materials necessary for the performance of the wargame |
| Pre-workshop Prep | *in*Thought team members will conduct onsite preparations either the day of or day prior to the workshop |
| Workshop Execution | *inThought* will provide a report of the key findings and summaries from the workshop discussions one week after the workshop completion |

# Conference Coverage Capabilities

# *in*Thought's Unique Conference Coverage Technology

In this uncertain environment, conventions and other events are taking precautions amid the COVID-19 outbreak. Some conferences are postponing, while others are providing virtual access. Many companies are restricting travel for employees, preventing them from attending conferences. In addition, many speakers are unable to attend as several leading academic institutions have restricted physician travel for months.

inThought's web-based technology platform, inVision, can help clients **stay on top of cutting-edge scientific information and presentations, in this fluid situation**.

inVision's Conference Module is uniquely suited for biopharma companies to access relevant congress data and analysis, even if they are not attending the convention onsite. Available for an in-person or virtual conference, the system provides organized access to slide decks, presentation notes, and Q&A highlights. Data is maintained on your customized project site for an unlimited number of users. Since the COVID-19 outbreak, we have been adding **new functionality to inVision to accommodate virtual conferences and virtual debriefs**.

inThought can cover the conference for you, or we can work with your current vendor to use only the technology platform.

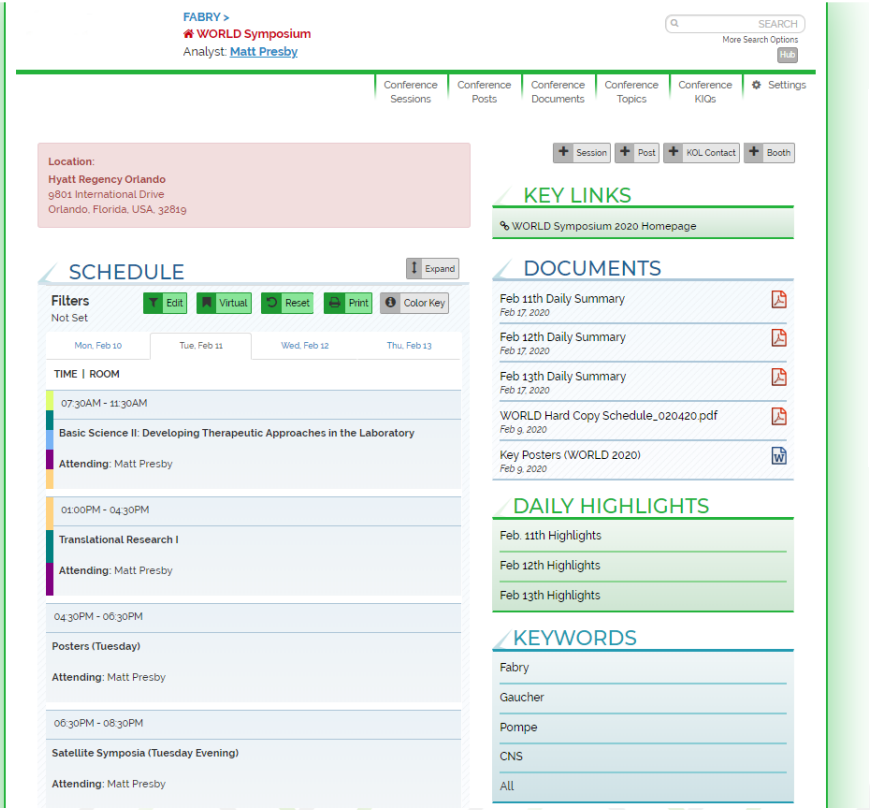
# Conference Coverage
## *Customized inVision Homepage*

*The inVision conference homepage is customized based on the client's key intelligence themes (KITs) and key intelligence questions (KIQs).*

### Overview

- Conference coverage schedules are customized based on client keywords and KIQs
- Schedules are uploaded to the inVision platform to allow the inThought analyst to capture presentation/poster images and notes
- Attending internal client members can also access inVision to input their own notes and images
- Presentations/posters are organized by key themes on inVision

### inVision Conference Layout




### Key Themes

**KEYWORDS**

- Fabry
- Gaucher
- Pompe
- CNS
- All

**COMPETITORS**

- Abeona
- Audentes
- Freeline
- Denali

Topic folders to organize presentations by theme



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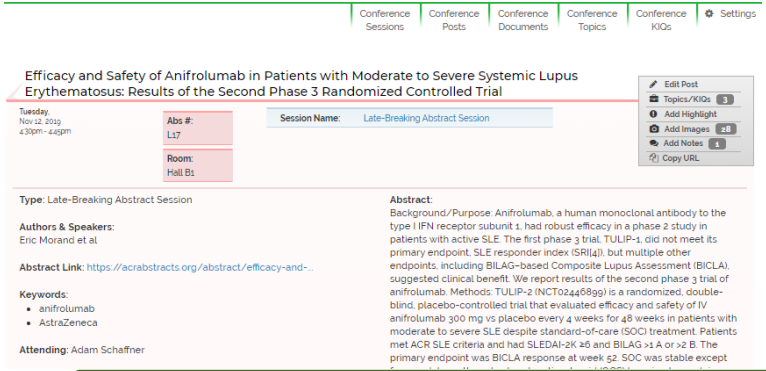
# Conference Coverage
## *Session / Poster Overview*

*Conference coverage is centralized on inVision, allowing both inThought analysts and internal client team members to simultaneously contribute images, notes, and view schedules.*

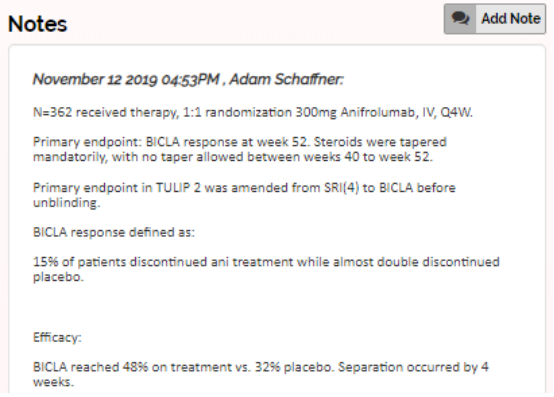
**Overview**

- Posts are color coded based on importance and tagged to key themes
- The analyst's notes and images are compiled in one central location within the relevant session
- Theses notes/images can be searched on inVision by keyword, priority, and type
- Team members can view the entire post, review images, and download or email images to colleagues

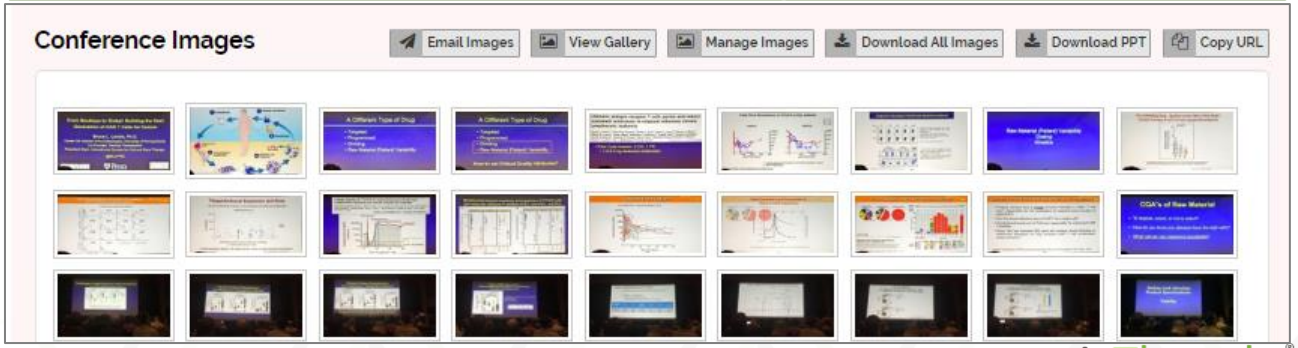
**Session Title / Speaker / Time / Location**




**Analyst Analysis**




**Presentation / Poster Images**



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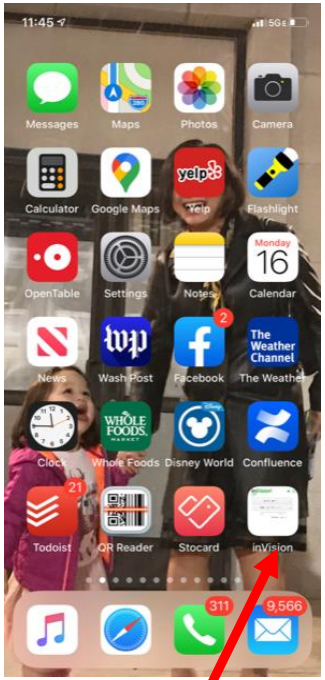
# Conference Coverage on Mobile Device

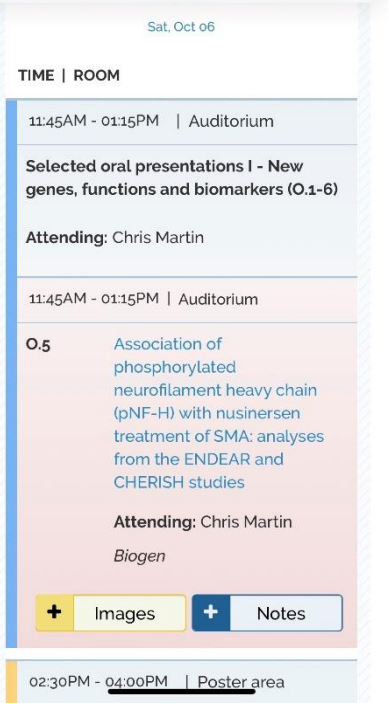
*The inVision conference coverage on a mobile device is similar to the web-based layout. Notes can be directly drafted in inVision or cut and pasted from other apps, such as Notes. Images can be taken in inVision or added through photos on the mobile device.*

**Icon on mobile homepage**

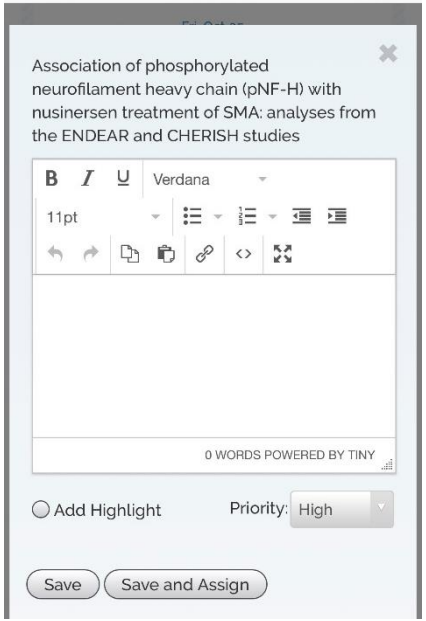



inVision icon

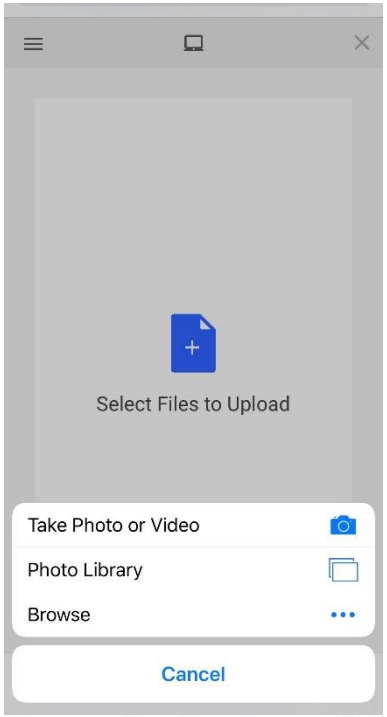
**Mobile layout**




**Notes function**




**Adding images**



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# Conference Coverage

## *Booth Coverage*

*Booth coverage allows the inThought analyst to capture key messaging themes that are stored on inVision.*

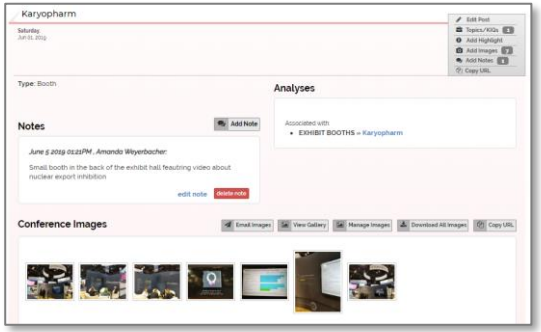
### Photos

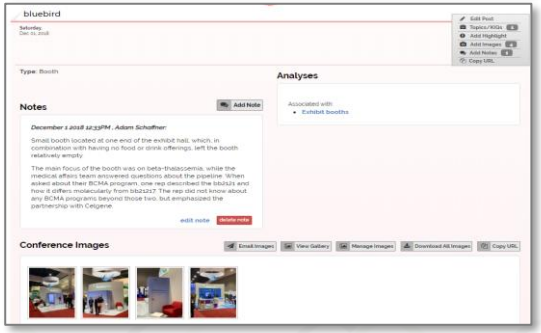


### Commentary

- Boehringer Ingelheim has 3 booths. One main booth focused on Spiriva and it is also one of the largest in the hall.
- A mist from a large Respimat inhaler flows in the booth.
- Key messaging highlighted that *"50% of patients remain symptomatic" and promotes add on TIO."*
  - *"Spiriva significantly reduced the risk of asthma worsening - 31% risk reduction"*
  - *"Stay in front of asthma"*
  - *"New Spiolto Respimat for further improvement beyond Spiriva Respimat"*
- Even the floor is a video of growing tree roots and huge curved screens rise from this presenting information on Spiolto. The metaphor is that Spiriva was the roots. A key message of Spiolto is "An advance in COPD care built on strong roots".
- Messaging on the screens focused on the rapid onset and improvement in LF compared to Spiriva.
- A wall focused on Respimat had a screen highlighting a study Respimat with other devices (TurboHaler etc.) and this indicated a > 50% deposition to the lungs, much better than any other device. The booth also highlights all of the drugs available with Respimat (Spiriva, Spiolto, Striverdi, CombiVent, etc.) Respimat is the only inhaler promoted.
- 30-35% of the booth focused on IPF.

### *in*Vision Cloud Storage



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# Conference Coverage

## *Key Intelligence Questions (KIQs)*

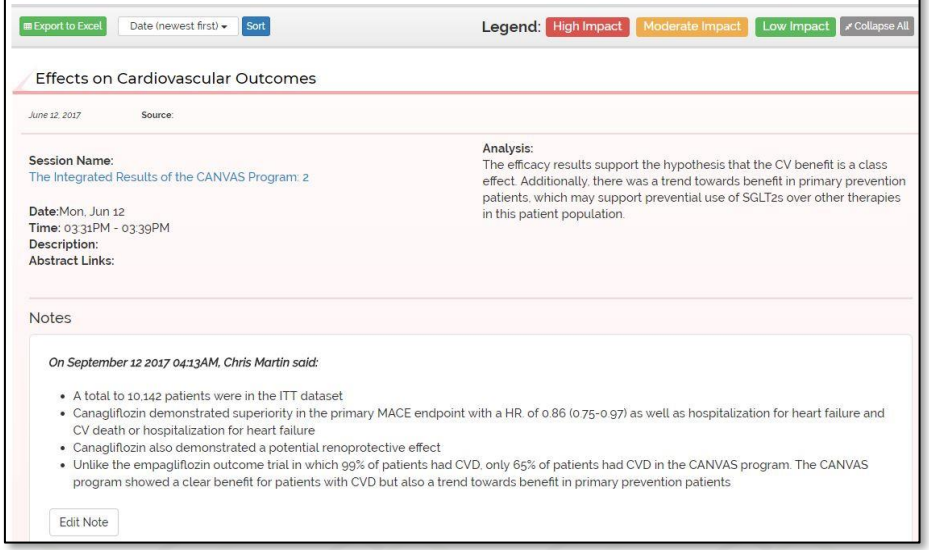
*in*Vision *maintains a repository of client's KIQs and relevant answers.*

### KEY INTELLIGENCE QUESTIONS

| Question | Answer |
|---|---|
| Are the results seen in the EMPA-REG OUTCOME trial considered a SGLT-2 class effect? | The CVOT results in the canagliflozin CANVAS trial, which were similar to the empagliflozin CVOT results, suggest that the CV benefits seen in both trials may be viewed as a SGLT2 inhibitor class... |
| How does the diabetes community think about changing CVOTs? | Potential changes include: using a lower risk population including primary prevention patients, longer follow-up, modifying endpoints including including heart failure as a primary endpoint and the... |
| How will CVOTs affect the prescribing of diabetes drugs? | Presenters recommended treating diabetes patients with CVD or high CVD risk as a specific category when choosing 2nd line therapy by prescribing a diabetes drug which as shown a CVD risk reduction... |
| How will diabetes drug CVOTs affect guidelines? | Presenters advocated changing ADA guidelines to include diabetes drug CVOTs and CV risk in the choice of a second line therapy after metformin. |

### How will diabetes drug CVOTs affect guidelines?

**Answer:** *Presenters advocated changing ADA guidelines to include diabetes drug CVOTs and CV risk in the choice of a second line therapy after metformin.*



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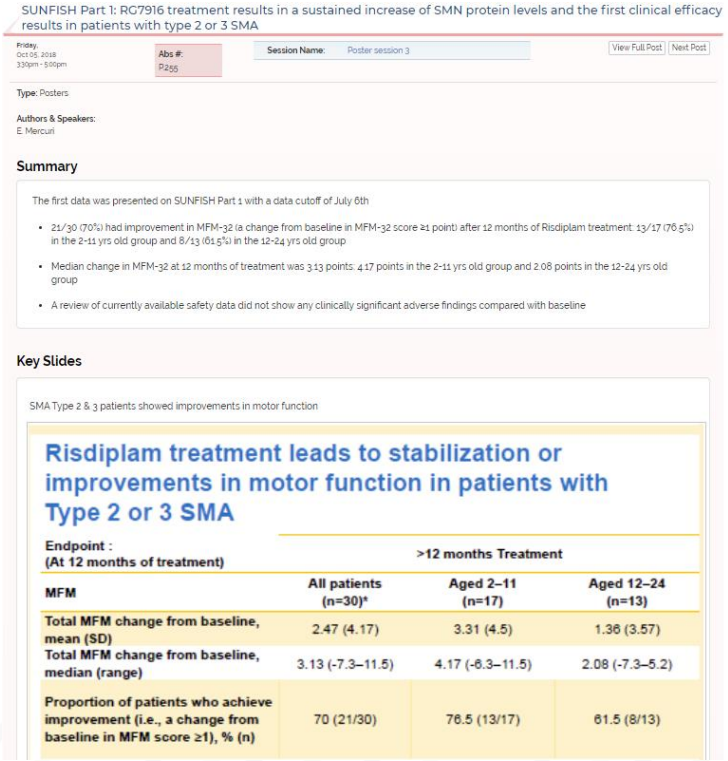
# Virtual Conference Coverage

*The inVision conference site has features enabling coverage of virtual conferences. These features also allow for attending conferences "virtually" as well as conducting virtual conference debriefs*

## Overview

- inVision provides **links to virtual presentations**, whether the conference is virtual or if team members are attending "virtually."
- The **debrief function** allows the selection of key information, including pictures of data, to be combined with summary text and analysis in a virtual debrief. Insights from the debrief can be incorporated into the conference project.
- A **virtual conference tour** can be constructed with key summaries and pictures to provide a quick summary of conference highlights with the ability to delve into more detailed information

## Virtual Debrief/Conference Summary



SUNFISH Part 1: RG7916 treatment results in a sustained increase of SMN protein levels and the first clinical efficacy results in patients with type 2 or 3 SMA

Friday, Oct 05, 2018 3:30pm - 5:00pm

Abs #: P255

Session Name: Poster session 3

View Full Post | Next Post

Type: Posters

Authors & Speakers: E. Mercuri

**Summary**

The first data was presented on SUNFISH Part 1 with a data cutoff of July 6th

- 21/30 (70%) had improvement in MFM-32 (a change from baseline in MFM-32 score ≥1 point) after 12 months of Risdiplam treatment: 13/17 (76.5%) in the 2-11 yrs old group and 8/13 (61.5%) in the 12-24 yrs old group
- Median change in MFM-32 at 12 months of treatment was 3.13 points: 4.17 points in the 2-11 yrs old group and 2.08 points in the 12-24 yrs old group
- A review of currently available safety data did not show any clinically significant adverse findings compared with baseline

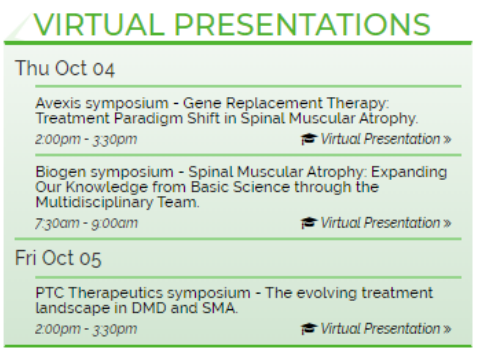
**Key Slides**

SMA Type 2 & 3 patients showed improvements in motor function

**Risdiplam treatment leads to stabilization or improvements in motor function in patients with Type 2 or 3 SMA**

| Endpoint : (At 12 months of treatment) | >12 months Treatment | | |
|---|---|---|---|
| MFM | All patients (n=30)* | Aged 2–11 (n=17) | Aged 12–24 (n=13) |
| Total MFM change from baseline, mean (SD) | 2.47 (4.17) | 3.31 (4.5) | 1.36 (3.57) |
| Total MFM change from baseline, median (range) | 3.13 (-7.3–11.5) | 4.17 (-6.3–11.5) | 2.08 (-7.3–5.2) |
| Proportion of patients who achieve improvement (i.e., a change from baseline in MFM score ≥1), % (n) | 70 (21/30) | 76.5 (13/17) | 61.5 (8/13) |

## Virtual Presentations



VIRTUAL PRESENTATIONS

Thu Oct 04

Avexis symposium - Gene Replacement Therapy: Treatment Paradigm Shift in Spinal Muscular Atrophy.
2:00pm - 3:30pm — Virtual Presentation »

Biogen symposium - Spinal Muscular Atrophy: Expanding Our Knowledge from Basic Science through the Multidisciplinary Team.
7:30am - 9:00am — Virtual Presentation »

Fri Oct 05

PTC Therapeutics symposium - The evolving treatment landscape in DMD and SMA.
2:00pm - 3:30pm — Virtual Presentation »



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# Conference Coverage
## *Daily Debrief Example*

American Society of Hematology
Helping hematologists conquer blood diseases worldwide

**December 8th Highlights**

Sunday highlights from ASH included NHL data on the combination of obinutuzumab/lenalidomide and the Genentech/Roche CD20:CD3 bispecific mosunetuzumab (Plenary Session).

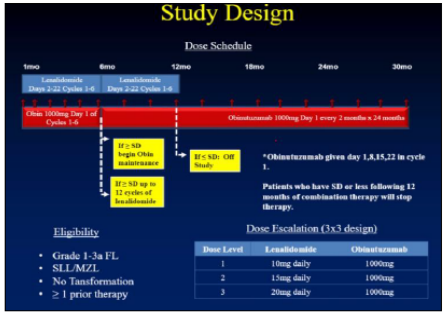
***Indolent non-Hodgkin Lymphoma***

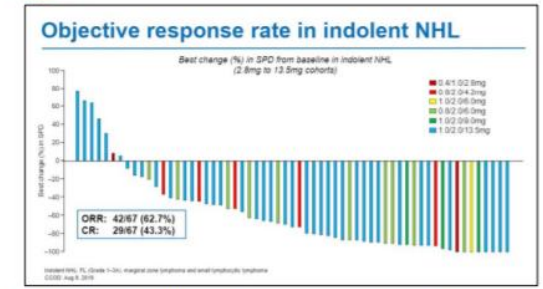
**Abs. 348: A Phase I/II Study of Lenalidomide Plus Obinutuzumab in Relapsed Indolent Lymphoma**

**Nathan Fowler (MD Anderson Cancer Center)** gave a morning presentation on a Phase 1/2 trial studying lenalidomide plus obinutuzumab in relapsed indolent lymphoma patients. 86% of enrolled patients had FL (n=57) with a minority of MZL and SLL patients also under study. The majority of patients were on their 3rd line of therapy or greater. Very impressive responses were observed in FL with an ORR of 100% (CR rate 75%) and 74% of patients experiencing PFS at 24 months. For the overall indolent NHL population the OS at 24 months was 94%. Additionally, the following subset analyses were provided for the overall indolent NHL population:

- POD24: ORR = 96% (CR rate 66%)
- ≥3 lines: ORR = 97% (CR rate 68%)
- Rituximab refractory: ORR = 100% (CR rate = 63%)

The toxicity profile was manageable with the most common Grade 3-4 AEs being neutropenia (21%), infection (14%), and thrombocytopenia (11%).

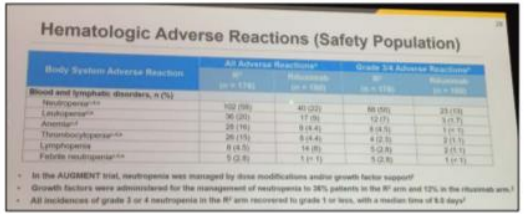



**Product Theater: The Role a New Combination Therapy in Previously Treated Follicular Lymphoma**

Sponsor: Celgene

Attendance was moderate for this Celgene product theater, with only about 50% of seats filled. During this talk the AUGMENT trial data assessing the combination of lenalidomide/rituximab ($R^2$) in previously treated FL was reviewed. Key commentary from the presenter mentioned that neutropenia was easily managed with dose modifications and growth factor support. It was noted that the associated neutropenia was not necessarily considered a direct toxicity but more of a maturation risk. Further, the presented commented that, "*there was not much in the way of Grade 3-4 AEs with this treatment*." No new efficacy or safety data was presented.



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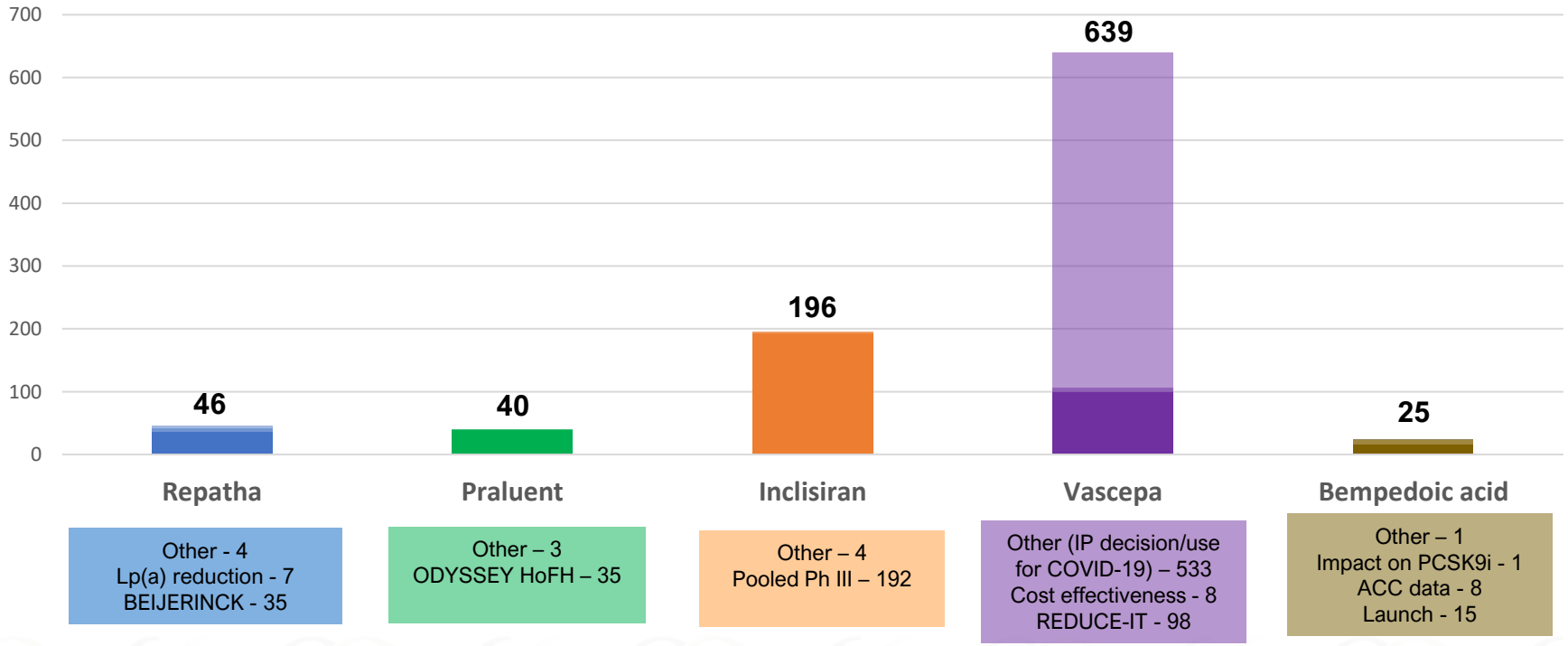
# Social Media Monitoring

## Sample Work

# Drug-Specific Social Media Metrics

*Tailoring the information monitored to your needs allows you to better understand your overall market.*

## Overall Tweet Volume During ACC 2020*




* compiled from social media feed using Meltwater



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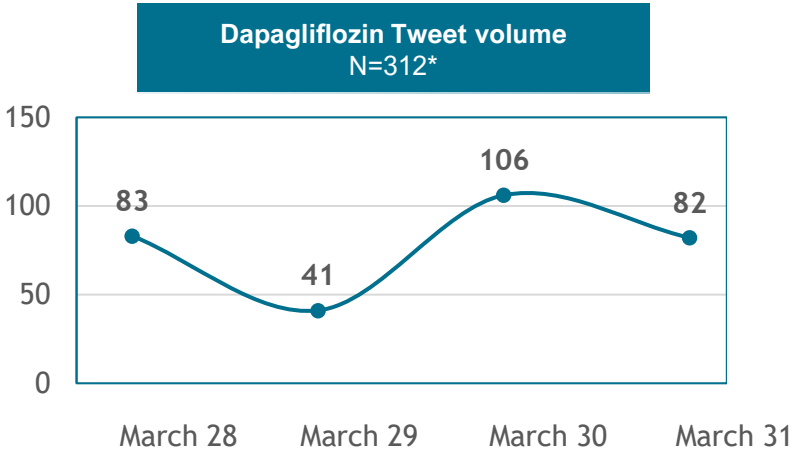
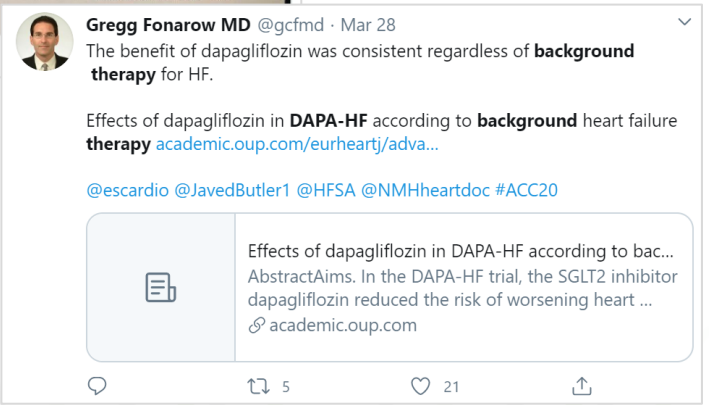
# Social Media Details by Drug

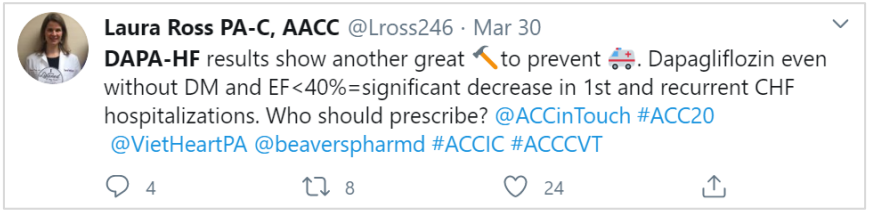
*During medical meetings, following data volume by day as well as what is being said can allow you to understand how your data are being received and stay on top of competitors.*




## ACC 2020 Dapagliflozen Key Tweets

*Twitter activity was largely neutral or positive*


* compiled from social media feed using Meltwater

# *in*Thought Contact Information

**Ben Weintraub, PhD**
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1.646.331.9234

After completing his training in immunology and biochemistry, Dr. Weintraub began work as a financial analyst in 2000. Collaborating with Dr. Henderson, Dr. Weintraub co-founded BiotechTracker, an online tool for investors. In 2004, he became a licensed security analyst with Hibernia Southcoast Capital covering the biotechnology sector, and later performed the same role at Variant Research. In 2006, Dr. Weintraub joined Dr. Henderson and Dr. Zuckerman at Reuters Insight, providing analysis of drug development and trends in medicine to professional investors. Dr. Weintraub's team moved to inThought in January 2009. Through a divestiture by Wolters Kluwer, inThought and Source Healthcare Analytics became part of Symphony Health Solutions, a healthcare information company. inThought Research LLC formed a stand-alone company in May 2014.

Prior to 2000, Dr. Weintraub was senior scientific editor for the biology research journals Cell and Molecular Cell. Dr. Weintraub performed biochemistry and immunology research at Stanford University and at the John Curtin School of Medical Research in Canberra, Australia. He earned his doctorate in Biology from the University of California, San Diego, and a Bachelor of Science in Life Science from the Massachusetts Institute of Technology.

**Adam Schaeffer, MBA**
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1.301.602.3297

Mr. Schaeffer is a founding partner of inThought Research. He is responsible for identifying and developing new business opportunities, as well as expanding the presence of the company and its brand. Mr. Schaeffer has over 20 years of experience in healthcare industry consulting.

Prior to inThought, he served as Director of Institutional Sales at Symphony Health Solutions where he oversaw key pharmaceutical accounts and launched the Wall Street practice. Mr. Schaeffer also served as Business Development Manager at Wolters Kluwer Health. In this role, he helped to build an independent research group and life science consulting business. He was previously US Director of Sales and Marketing at Informa. Mr. Schaeffer received his Master in Business Administration from the Robert H. Smith School of Business and has a Bachelor of Arts in Sociology from the University of Maryland.

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# *in*Thought Contact Information

**Doug Foster**
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Doug Foster is focused on the development and improvement of the inVision system. An MIT graduate, Doug brings a unique combination of entrepreneurial experience, knowledge and learning management system knowledge, and enterprise system integration experience. Before joining inPhronesis, Doug led teams at InterSystems and Boston Scientific, where he was responsible for implementing and managing learning and knowledge management systems for employees, partners and customers for as many as 25,000 users globally. Previously, Doug was the VP of Services for Click2Learn (now SumTotal Systems) where he led a team responsible for the development of custom eLearning for clients including Fidelity and Microsoft.

**Chris Martin, MSc, MBA**
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1.703.725.5820

Mr. Martin has over 18 years of experience in healthcare industry consulting, finance, and healthcare policy. He previously served in health policy roles at the White House Office of Management and Budget. These roles included Medicare Desk Officer for the Office of Information and Regulatory Affairs, where he was responsible for providing recommendations to senior White House policy officials on healthcare policies and regulations.

Mr. Martin was the Vice President of Research at Lese Investments LLC, a healthcare focused investment company, and co-portfolio manager at Cameron Capital. He has also performed consulting in the healthcare industry at Biotech Tracker and McKinsey and Company. Mr. Martin has a Master in Business Administration from Harvard Business School, a Master in Engineering from Villanova University, and a Bachelor of Science, with distinction, in Mechanical Engineering from Cornell University.

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# *in*Thought Contact Information

**Amanda Weyerbacher, PhD**
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Prior to joining *in*Thought, Dr. Weyerbacher was a scientific consultant to biotechnology companies, providing scientific and regulatory analysis of compounds and distinct therapeutic combinations. She has previously worked as a Senior Scientist at L'Oreal Research and Development. In this role, she managed the clinical testing program of multiple consumer products and supported cross-functional teams with scientific leadership and expertise to facilitate blockbuster product launches.

Dr. Weyerbacher graduated from Skidmore College with a Bachelor's degree in Biology-Chemistry before receiving a PhD in Pharmacology from Weill Cornell Medical College. Her dissertation research focused on the identification of critical pain signaling proteins, cytokines and immune/central nervous system interactions as relevant pharmacological targets for clinical pain control. In between college and graduate school, Dr. Weyerbacher was a Clinical Research Study Assistant at Memorial Sloan Kettering Cancer Center. In this role, she managed an active clinical trial program, defining and monitoring project scope, timelines and deliverables from project initiation to close-out. She has presented her research in pharmacology, neuroscience and oncology at several scientific conferences. Dr. Weyerbacher is the immediate past President and active member of the MetroNY Chapter of the Association for Women in Science (AWIS).

**Adam Schaffner, PhD**
aschaffner@inthought.com
1.818.427.3385

Dr. Schaffner joined inThought in 2017 after earning his PhD in Biomedical Sciences at the Mount Sinai School of Medicine in New York, where he developed novel therapeutic strategies to target Parkinson's Disease and other neurodegenerative disorders.

While completing his dissertation, he worked as a venture intern at Celdara medical, where he conducted due diligence on academic innovations for partnership, development, and investment opportunities. Beyond the science, Dr. Schaffner has held various leadership roles as a student at Mount Sinai, and served as Co-founder and Co-chair of the Trainee Health and Wellness (THAW) Committee, which tackles systemic issues endangering the mental well-being of academic research trainees. He earned his Bachelor of Science in Biochemistry at the University of California, Los Angeles.

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# *in*Thought Contact Information

**Julie Hoggatt**
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1.504.220.9366

Ms. Hoggatt covers the medical technology, women's health, aesthetics and some infectious disease indications. Ms. Hoggatt comes from Noble Financial where she was the Senior Medical Technology analyst. Prior to Noble, Ms. Hoggatt helped start the independent research firm, Variant Research with Dr. Weintraub and Dr. Henderson.

Before Variant she worked at Hibernia Southcoast Capital, where she was also a Medical Technology Analyst and a Vice President of Equity Research. Ms. Hoggatt began in the investment industry as an associate equity analyst at Morgan Keegan & Company covering medical devices and earned the title Associate Vice President of Equity Research. Her professional career has been focused on the healthcare sector and has included in-depth coverage of over thirty different companies in the Medical Technology and Medical Device industries, allowing her to share a wealth of knowledge and insight with our clients. Ms. Hoggatt received her Bachelors of Business Administration and Master of Accountancy from Millsaps College in Jackson, Mississippi.

**Michelle Rivera, MBA, PhD**
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1.917.575.1372

Dr. Rivera joined inThought in early 2019. She completed her PhD research at Columbia University Medical Center in Pathobiology and Molecular Medicine. Her work at the Center for Motor Neuron Biology and Disease focused on drug development for the neuromuscular pediatric disorder, Spinal Muscular Atrophy (SMA).

Prior to Columbia, Dr. Rivera was a research associate at Cold Spring Harbor Laboratories, where she specialized in molecular research of pre-mRNA splicing mechanisms. She also earned an MBA in Finance and a Master of Science in Biophysics from the University of Barcelona.

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# *in*Thought Contact Information

**Roshni Basu, PhD**
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Dr. Basu joined inThought in 2015, after completing her post-doctoral studies in Immunology at Memorial Sloan-Kettering Cancer Center, where she studied mechanisms potentiating cytotoxic T cells. While at MSKCC, she also worked as a Life Science Analyst for a boutique consulting firm, providing due diligence on diverse therapeutic areas to hedge funds and mutual funds.

In addition to research, Dr. Basu has regularly been involved in several public health and science education projects in the New York City area. She earned her PhD in Cell Biology from Columbia University in New York and a B.Sc. in Biology from McGill University in Montréal.

**Samantha Crofskey, BSc**
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1.732.455.9121

Ms. Crofskey has over 12 years of experience in medical writing, account management and pharmaceutical industry consulting. She provides coverage for the immunology and neurology sectors, including everything from emerging targets and drug development to market analysis and customized business intelligence.

Prior to joining inThought, Ms. Crofskey worked as a medical writer for Wolters Kluwer in Auckland, New Zealand before moving to London to work as an Editorial Project Manager at Current Medicine Group, a Springer Healthcare company, and later, as an Editorial Project Manager at BioScience Communications. Ms. Crofskey returned to Wolters Kluwer in 2010 and subsequently joined the inThought team as a consultant in 2011. Ms. Crofskey earned her Bachelor of Science in Neuroscience and Pharmacology from the University of Otago in New Zealand.

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# *in*Thought Contact Information

**Matt Presby, PhD**
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1.732.690.4119

Dr. Presby joined inThought in 2019. Previously, he worked for a boutique biopharma consulting firm and also completed an internship in equity research at Leerink Partners. In these roles, he has developed experience in business development assessments, forecasting, and market access.

Dr. Presby received his Ph.D. in Immunology from the Johns Hopkins School of Medicine where his thesis work focused on the discovery of biomarkers in autoimmune disease and T Cell functionality. Additionally, while at Hopkins he was a co-founder of the Johns Hopkins Biotech Investment Group (JHBIG) which focuses on preparing students for careers in equity research and venture capital. He earned his B.S. in Biology from Gettysburg College.

**Lavan Khandan, PhD**
lkhandan@inthought.com
1.860.965.8007

Dr. Khandan joined *in*Thought Research in 2019. He has held previous positions as a Fellow at a life science consulting firm and as a Scientist at Editas Medicine. In these roles, Dr. Khandan built expertise in asset scouting, evaluating rare diseases and therapeutic areas, and building market access and commercial viability assessments.

Dr. Khandan received his Ph.D. in Molecular, Cellular, and Developmental Biology from the University of Colorado, Boulder. His doctoral research focused on signaling and growth factors governing retinal vascularization. He has extensive experience in CRISPR/Cas9 gene editing, stem cell differentiation, and cell therapies. Dr. Khandan received his A.B. in Chemistry from Dartmouth College.

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