Developing Highly Differentiated Antibody Therapeutics

argenx

November, 2018
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Rapidly Emerging Leadership in Immunology
Pioneering differentiated therapeutic antibodies in severe autoimmune diseases and cancer

1. Novel Target Biology
   - Integrated via advanced technology suite
   - First- and best-in-class potential

2. Innovative Access Program
   - Robust science
   - Collaborative
   - Efficient pipeline expansion

3. Highly Productive Development Engine
   - Rapid development timeline
   - ~New candidate each year since 2009

4. Maximum Value per Asset
   - Pipeline-in-a-product strategy
   - Strong biological rationale

Translate immunology breakthroughs into novel medicines which truly impact patients’ lives, in a sustainable value creating approach
Strong Momentum with Important Near-Term Catalysts

2018 a monumental year with **three** additional data milestones before YE

### 2018 Accomplishments To-Date

**ARGX-113 efgartigimod**
- MG: Phase 2 data - AAN
- MG: End-of-Phase 2 meeting – FDA/PMDA
- MG: Phase 3 study
- ITP: Phase 2 topline data
- PV: Interim Phase 2 data
- SC formulation: Phase 1 data
- CIDP: 4th indication

**ARGX-110 cusatuzumab**
- AML: Ongoing enrollment in Phase 2 trial
- AML: Phase 1 dose-escalation data (ASH abstract)

**ARGX-117**
- Exercised license option

### Upcoming Milestones

**ARGX-113 efgartigimod**
- ITP: Phase 2 full data at ASH18
- ITP: End-of-Phase 2 meeting - FDA
- ITP: Phase 2 subcutaneous study initiation (1H19)
- ITP: Phase 3 initiation (2H19)
- PV: Phase 2 full data (1H19)
- CIDP: Phase 2 initiation (1H19)

**ARGX-110 cusatuzumab**
- AML: Phase 1 dose-escalation full data at ASH18
- AML: Phase 2 full data in 2H19
- CTCL: Phase 2 full data at ASH18

**ARGX-117**
- R&D-day: full preclinical update and first indication

Continued Progress Across Partnered Programs including Option Exercise by AbbVie for ARGX-115
# Deep Pipeline of Wholly-Owned Candidates for Orphan Indication

Significant partner activity across other therapeutic areas

<table>
<thead>
<tr>
<th>Product Candidate</th>
<th>Target</th>
<th>Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>BLA</th>
<th>Next Milestone / Commentary</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Wholly-Owned Product Candidates</strong></td>
<td></td>
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<tr>
<td>ARGX-113 (efgartimod)</td>
<td>FcRn</td>
<td>Myasthenia Gravis</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>3Q18: Phase 3 initiated</td>
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<tr>
<td></td>
<td></td>
<td>Immune Thrombocytopenia (“ITP”)</td>
<td></td>
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<td></td>
<td></td>
<td></td>
<td>ASH18: Detailed Phase 2 data</td>
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<td></td>
<td></td>
<td>ITP Subcutaneous Formulation</td>
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<td></td>
<td></td>
<td></td>
<td>1H19: Phase 2 initiation in subcutaneous formulation</td>
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<td></td>
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<td>Pemphigus Vulgaris</td>
<td></td>
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<td></td>
<td></td>
<td></td>
<td>1H19: Phase 2 topline data</td>
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<tr>
<td></td>
<td></td>
<td>Chronic Inflammatory Demyelinating Polyneuropathy</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>1H19: Phase 2 initiation</td>
</tr>
<tr>
<td>ARGX-110 (cusatuzumab)</td>
<td>CD70</td>
<td>T-Cell Lymphoma</td>
<td></td>
<td></td>
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<td></td>
<td></td>
<td>ASH18: Phase 2 topline results CTCL</td>
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<tr>
<td></td>
<td></td>
<td>Acute Myeloid Leukemia</td>
<td></td>
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<td></td>
<td></td>
<td></td>
<td>ASH18: Phase 1 full data in AML/MDS</td>
</tr>
<tr>
<td>ARGX-117</td>
<td>Novel complement target</td>
<td>Severe Autoimmune Diseases</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Antibody-mediated autoimmune diseases Complementary to ARGX-113</td>
</tr>
<tr>
<td><strong>Partnered Product Candidates</strong></td>
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<tr>
<td>ARGX-112</td>
<td>IL-22R</td>
<td>Skin Inflammation</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Eligible for up to ~€100mm in milestones and tiered royalties</td>
</tr>
<tr>
<td>ARGX-115</td>
<td>Cancer Immunotherapy</td>
<td>AbbVie exercised option to develop and commercialize in August 2018</td>
<td></td>
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<tr>
<td>ARGX-116</td>
<td>ApoC3</td>
<td>Dyslipidemia</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Received $60mm so far; eligible for up to $625mm milestones &amp; tiered royalties</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Eligible for double-digit royalties and exclusive option to license the program</td>
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</tr>
</tbody>
</table>

- **Innovative Access Program**: 7 live programs
- Antibody discovery alliance with Shire focused on multiple rare disease targets – 2 options exercised
- Additional programs include ARGX-111, targeting c-MET in solid tumors and blood cancers (P1 concluded, wholly-owned, available for partnering), and ARGX-109 (gerilimzumab), targeting IL-6 for rheumatoid arthritis (P1 concluded, partnered with Genor Biopharma)
Efgartigimod: A Pipeline-in-a-Product Opportunity
• Human IgG1 Fc-fragment that utilizes ABDEG™ Fc engineering technology\(^{(2)(3)(4)}\)
• Targets and binds to FcRn blocking recycling of IgG leading to elimination of IgG antibodies\(^{(3)(4)}\)
• Cannot engage Fc\(\gamma\) receptors when bound to its target FcRn\(^{(3)(4)}\)
• Pathogenic IgG antibodies mediate multiple autoimmune diseases

\(^{(1)}\) Roopenian et al. 2007, Nat Rev Immunol.
\(^{(3)}\) Ulrichts et al. 2018, JCI
\(^{(4)}\) argenx data
Novel Modality in Severe Autoimmune Diseases

Common Characteristics of Beachhead Indications for efgartigimod

- Pathogenic auto-antibodies causal to disease biology
- Common treatments include corticosteroids, broad immunosuppressants, IVIG, plasmapheresis, Rituxan – with mixed response rates and serious side effects
- Orphan potential in United States (MG: 50-60K\(^{(1)}\); ITP: 50K\(^{(2)}\); PV: 30-40K\(^{(3)}\))
- Potential pharmacoeconomic benefit to healthcare system given price of targeted therapies (e.g., Soliris for refractory MG ~$700K / year\(^{(4)}\))

### Myasthenia Gravis
- Block Acetylcholine Receptors
- Cross-link + internalize AChRs
- Complement recruitment

### Immune Thrombocytopenia
- Enhance platelet clearance
- Platelet killing
- Inhibit platelet production
- Reduced platelet function

### Pemphigus Vulgaris
- Sterically hinder epithelial adhesion affecting skin and mucosal integrity

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(2) Wall street research; Estimated 65K ITP patients in US with ~80% diagnosed with primary ITP
(3) IPPF (www.pemphigus.org)
(4) Source: Reprinted with permission by First Databank Inc.; WAC = Wholesale Acquisition Cost 8/21/17
**MG Phase 2 Trial: Completed, Proof of Concept Established**

### Key inclusion criteria
- Generalized MG patients
- MGFA Class II, III, or IVa
- Positive for anti-AChR auto-antibodies
- MG ADL score of ≥ 5 at screening(*)
- On a stable dose of their SoC

### Treatment Phase
- **SoC + ARGX-113 (10mg/kg)**
  - N=12
  - 4 doses; N= 24

### Follow-up Phase
- Study start-to-finish in 11 months

### Primary endpoint
- Safety & Tolerability

### Secondary endpoints
- **Efficacy**
  - (MG-ADL; QMG; MGC; MG-QoL)
- **PK**
- **PD**
  - total IgG; pathogenic IgG
- **Immunogenicity**

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(*) >50% of the score attributed to non-ocular items

Clinicaltrials.gov: NCT029655573, argenx data
Efgartigimod Safety and Tolerability Data from Phase 2

2 hour infusion enabling out-patient administration

- Efgartigimod was well-tolerated in patients; confirmed findings from Phase 1 healthy volunteer trial
- The TEAEs profile was balanced between efgartigimod and placebo
- TEAEs were mostly mild (grade 1) in severity; no severe AEs were reported
- No deaths, serious AEs or TEAEs leading to discontinuation of treatment were reported during the trial

<table>
<thead>
<tr>
<th>Treatment Emergent Adverse Events (TEAEs) Reported in ≥ 2 patients</th>
<th>Placebo (N = 12)</th>
<th>Efgartigimod (N = 12)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>TEAEs (Total)</strong></td>
<td>10 (83.3%)</td>
<td>10 (83.3%)</td>
</tr>
<tr>
<td>• Headache</td>
<td>3 (25.0%)</td>
<td>4 (33.3%)</td>
</tr>
<tr>
<td>• Nausea</td>
<td>1 ( 8.3%)</td>
<td>1 ( 8.3%)</td>
</tr>
<tr>
<td>• Diarrhea</td>
<td>1 ( 8.3%)</td>
<td>1 ( 8.3%)</td>
</tr>
<tr>
<td>• Abdominal pain upper</td>
<td>1 ( 8.3%)</td>
<td>1 ( 8.3%)</td>
</tr>
<tr>
<td>• Arthralgia</td>
<td>2 (16.7%)</td>
<td>-</td>
</tr>
<tr>
<td>• B-lymphocyte decrease</td>
<td>-</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Lymphocyte count decrease</td>
<td>-</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Monocyte count decrease</td>
<td>-</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Neutrophil count increase</td>
<td>-</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Myalgia</td>
<td>-</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Pruritus</td>
<td>2 (16.7%)</td>
<td>1 ( 8.3%)</td>
</tr>
<tr>
<td>• Rhinorrhea</td>
<td>1 ( 8.3%)</td>
<td>1 ( 8.3%)</td>
</tr>
<tr>
<td>• Tooth abscess</td>
<td>2 (16.7%)</td>
<td>-</td>
</tr>
<tr>
<td>• Toothache</td>
<td>2 (16.7%)</td>
<td>-</td>
</tr>
<tr>
<td><strong>Efgartigimod deemed related TEAEs</strong></td>
<td>3 (25.0%)</td>
<td>8 (66.7%)</td>
</tr>
<tr>
<td>• Headache</td>
<td>1 ( 8.3%)</td>
<td>3 (25.0%)</td>
</tr>
<tr>
<td>• Monocyte count decrease</td>
<td>0 ( 0.0%)</td>
<td>2 (16.7%)</td>
</tr>
<tr>
<td>• Rhinorrhea</td>
<td>1 ( 8.3%)</td>
<td>1 ( 8.3%)</td>
</tr>
</tbody>
</table>
### MG Phase 2 Baseline Population and Disease Characteristics

<table>
<thead>
<tr>
<th></th>
<th>Placebo (N = 12)</th>
<th>Efgartigimod (N = 12)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Age (mean ± SD)</strong></td>
<td>43.5 ± 19.3</td>
<td>55.3 ± 13.6</td>
</tr>
<tr>
<td><strong>Gender (N (%))</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Male</td>
<td>4 (33.3%)</td>
<td>5 (41.7%)</td>
</tr>
<tr>
<td>Female</td>
<td>8 (66.7%)</td>
<td>7 (58.3%)</td>
</tr>
<tr>
<td><strong>Race</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Asian</td>
<td>-</td>
<td>1 (8.3%)</td>
</tr>
<tr>
<td>Black / African American</td>
<td>1 (8.3%)</td>
<td>-</td>
</tr>
<tr>
<td>White</td>
<td>11 (91.7%)</td>
<td>11 (91.7%)</td>
</tr>
<tr>
<td><strong>MGFA Disease Class at Screening</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Class II</td>
<td>7 (58.4%)</td>
<td>6 (50.0%)</td>
</tr>
<tr>
<td>Class III</td>
<td>4 (33.3%)</td>
<td>6 (50.0%)</td>
</tr>
<tr>
<td>Class IV</td>
<td>1 ( 8.3%)</td>
<td>-</td>
</tr>
<tr>
<td><strong>Baseline QMG score (mean ± SD) (min, median, max score)</strong></td>
<td>11.8 ± 5.4 (3, 12.5, 24)</td>
<td>14.5 ± 6.3 (6, 14, 30)</td>
</tr>
<tr>
<td><strong>Baseline MG-ADL score (mean ± SD) (min, median, max score)</strong></td>
<td>8.0 ± 2.2 (5, 8, 13)</td>
<td>8.0 ± 3.0 (5, 7.5, 15)</td>
</tr>
<tr>
<td><strong>Baseline MGC score (mean ± SD)</strong></td>
<td>14.5 ± 4.5</td>
<td>16.7 ± 8.7</td>
</tr>
<tr>
<td><strong>Baseline MGQoL score (mean ± SD)</strong></td>
<td>14.5 ± 6.1</td>
<td>19.7 ± 5.7</td>
</tr>
<tr>
<td><strong>SoC</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Acetylcholinesterase inhibitors N (%)</td>
<td>11 (91.7%)</td>
<td>12 (100.0%)</td>
</tr>
<tr>
<td>Corticosteroids N (%)</td>
<td>5 (41.7%)</td>
<td>8 (66.7%)</td>
</tr>
<tr>
<td>Immunosuppressants N (%)</td>
<td>2 (16.7%)</td>
<td>9 (75.0%)</td>
</tr>
</tbody>
</table>
• PD effect of efgartigimod in the Phase 2 clinical trial very similar to the Phase 1 trial in healthy volunteers
• IgG reduction across IgG subtypes (AChR autoantibodies are IgG1/3; MuSK autoantibodies are IgG4)
• IgM, IgA and albumin levels not affected (data not shown)
Clinically Meaningful and Long-lasting Reduction of Efficacy Scores

- Clinically meaningful and statistically significant improvement reached in small patient population (N=24)
- Consistency between QMG and MG-ADL scores

**QMG**

**MG-ADL**

Weekly dosing, 4x total

(1) Quantitative Myasthenia Gravis
(2) Myasthenia Gravis Activity-of-Daily-Living
Robust Clinical Improvement over Placebo Group

- **Efgartigimod vs. placebo**: increasing differentiation observed with increasing MG-ADL/QMG thresholds

* Missing data point of 1 patient
Total & Pathogenic IgG Reduction Correlates with Clinical Improvements

Assessment for all efficacy scales

- Clinical improvement persists despite return of IgG levels
- Potential differentiation from PLEX, where clinical benefit was reported to be lost 2-4 weeks after end of treatment

(1) Kuks and Skallebaek, 1998, Transfus Sci
83% of patients treated with efgartigimod achieved a clinically meaningful response (MG-ADL ≥2)
75% of patients treated with efgartigimod had a clinically meaningful and statistically significant improvement in MG-ADL score for a period of at least 6 consecutive weeks versus 25% of patients on placebo
gMG: Conclusions of Phase 2 Study of Efgartigimod

- Consistent and favorable tolerability profile
- Fast, long-lasting and sustained benefit; clinically meaningful and statistically significant
- Strong correlation between IgG level reduction and disease improvement; supporting focus on IgG-mediated diseases
- Significant reduction of AChR autoantibodies
- Phase 2 execution advances efgartigimod into Phase 3 (initiated)
Myasthenia Gravis Phase 3 ADAPT Trial Design

Same Primary Endpoint as Successful Phase 2 Trial

- Randomized, double-blind, placebo-controlled, multicenter trial enrolling 150 patients in North America, Europe and Japan
- 10 mg/kg intravenous (IV) dose of efgartigimod over 26-week period
- Enrolling AChR positive and AChR negative patients with disease driven primarily by MuSK and LRP4 autoantibodies
- Patients in the ADAPT trial will be able to roll over into an open-label extension trial for a period of one year
- First patient dosed in September 2018
- Based on PMDA feedback, this Phase 3 trial, if data is positive, to also serve as a basis for Japan registrational submission

Primary endpoint
Myasthenia Gravis Activities of Daily Living (MG-ADL) Score

Secondary endpoints
Efficacy, Safety, Tolerability, Quality of Life and Impact on Normal Daily Activities Measures
## ITP Amended Phase 2 Trial Design

### Key inclusion criteria:
- ITP patients with platelet levels < 30 X 10^9/L
- On a stable dose of their SoC treatment prior to randomization

### Screening/Randomization

#### Main Study

<table>
<thead>
<tr>
<th>Treatment Phase</th>
<th>Follow-up Phase</th>
</tr>
</thead>
<tbody>
<tr>
<td>SoC + efgartigimod (10mg/kg) N=13</td>
<td></td>
</tr>
<tr>
<td>SoC + efgartigimod (5mg/kg) N=13</td>
<td></td>
</tr>
<tr>
<td>SoC + Placebo N=12</td>
<td></td>
</tr>
</tbody>
</table>

4 doses; N=38

#### Open Label Extension (OLE)

SoC + efgartigimod (10mg/kg) N = 12

33% of OLE patients come from placebo arm

### Primary endpoint

- **Safety & Tolerability**

### Secondary endpoints

- **Efficacy** (platelet counts, rescue therapy and bleeding)
- **PK**
- **PD** total IgG; pathogenic IgG
- **Immunogenicity**

### Timeline

- **≤2 weeks**
- **3 weeks**
- **21 weeks**
- **1 year**

**19 study centers from 8 countries**
ITP Amended Phase 2 Trial Design

Key inclusions criteria:
- ITP patients with platelet levels < 30 X 10^9/L
- On a stable dose of their SoC treatment prior to randomization

Main Study

Screening/Randomization | Treatment Phase | Follow-up Phase
--- | --- | ---

Key endpoints: Efficacy (platelet counts, rescue therapy and bleeding)

Safety & Tolerability

Open Label Extension (OLE)

- SoC + efgartigimod (10mg/kg) N = 12
- 33% of OLE patients come from placebo arm

Key Considerations
- Initiated appr. halfway through the study
- Only eligible for patients with platelet counts < 30x10^9/L (excl long responders still in response at end of study)
- 33% (N = 4) of OLE patients come from placebo arm
Efgartigimod Targets All Pathogenic AutoAb Actions Simultaneously
Potential to eliminate therapeutic cycling based on trial-and-error

1. Accelerate platelet clearance
   - Autoantibodies
   - Platelets
   - Macrophage
   - TPO-RA

2. Inhibit platelet production
   - Autoantibodies
   - Megakaryocyte

3. Induce platelet killing
   - Autoantibodies
   - Platelets

4. Interfere with platelet function
   - Autoantibodies
   - Platelets
   - Fibrinogen receptor
   - vWF receptor
   - Collagen receptor
## Baseline Population and Disease Characteristics

<table>
<thead>
<tr>
<th></th>
<th>Placebo (N = 12)*</th>
<th>Efgartigimod: 5mg/kg (N = 13)</th>
<th>Efgartigimod: 10 mg/kg (N = 13)**</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Age, median, (range)</strong></td>
<td>38.5 (19 - 69)</td>
<td>41.0 (22 - 77)</td>
<td>46.0 (29 - 62)</td>
</tr>
<tr>
<td><strong>Gender, N (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Male</td>
<td>5 (41.7)</td>
<td>4 (30.8)</td>
<td>9 (69.2)</td>
</tr>
<tr>
<td>• Female</td>
<td>7 (58.3)</td>
<td>9 (69.2)</td>
<td>4 (30.8)</td>
</tr>
<tr>
<td><strong>Race, N (%)</strong></td>
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<td></td>
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<tr>
<td>• White</td>
<td>11 (91.7)</td>
<td>12 (92.3)</td>
<td>13 (100)</td>
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<tr>
<td>• Not reported</td>
<td>1 ( 8.3)</td>
<td>1 ( 7.7)</td>
<td>-</td>
</tr>
<tr>
<td><strong>ITP Classification, N (%)</strong></td>
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<td></td>
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<tr>
<td>• Newly diagnosed (≤3 months)</td>
<td>-</td>
<td>2 (15.4)</td>
<td>-</td>
</tr>
<tr>
<td>• Persistent (&gt;3 and &lt;12 months)</td>
<td>3 (25.0)</td>
<td>1 ( 7.7)</td>
<td>4 (30.8)</td>
</tr>
<tr>
<td>• Chronic (≥12 months)</td>
<td>9 (75.0)</td>
<td>10 (76.9)</td>
<td>9 (69.2)</td>
</tr>
<tr>
<td><strong>Duration of ITP, median (range), years</strong></td>
<td>3.5 (0.3 - 47.8)</td>
<td>4.5 (0.1 - 34.2)</td>
<td>5.4 (0.7 - 28.7)</td>
</tr>
<tr>
<td><strong>Baseline platelet count, mean, /µL (range)</strong></td>
<td>18 (4 - 40)</td>
<td>18 (6 – 49)</td>
<td>15 (5 - 35)</td>
</tr>
<tr>
<td><strong>Baseline platelet count of &lt;15k/µL, N (%)</strong></td>
<td>6 (50.0)</td>
<td>7 (53.8%)</td>
<td>7 (53.8)</td>
</tr>
<tr>
<td><strong>SoC at baseline</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Corticosteroids N (%)</td>
<td>3 (25.0)</td>
<td>10 (76.9)</td>
<td>6 (46.2)</td>
</tr>
<tr>
<td>• TPOs N (%)</td>
<td>3 (25.0)</td>
<td>4 (30.8)</td>
<td>3 (23.1)</td>
</tr>
<tr>
<td>• Immunosuppressants N (%)</td>
<td>1 ( 8.3)</td>
<td>-</td>
<td>1 ( 7.7)</td>
</tr>
<tr>
<td>• Watch &amp; Wait N (%)</td>
<td>4 (33.3)</td>
<td>2 (15.4)</td>
<td>5 (38.5)</td>
</tr>
<tr>
<td>• Other N (%)</td>
<td>1 ( 8.3)</td>
<td>1 ( 7.7)</td>
<td>-</td>
</tr>
</tbody>
</table>

* Four placebo patients were discontinued before the end of the main study ** Two 10mg/kg patients were discontinued before receiving all 4 infusions argenx data
## Favorable Tolerability Profile

Consistent with efgartigimod clinical studies to date

- Well-tolerated profile: consistent with Phase 2 MG and Phase 1 healthy volunteer trials
- TEAEs profile balanced between efgartigimod and placebo arms
- TEAEs mostly mild (grade 1) in severity; one non-study drug related SAE (acute bronchitis)
- No deaths or TEAEs leading to discontinuation of treatment reported

<table>
<thead>
<tr>
<th>Treatment Emergent Adverse Events (TEAEs) Reported in ≥ 2 subjects (non-bleeding)</th>
<th>Placebo (N = 12)</th>
<th>Efgartigimod 5 mg/kg (N = 13)</th>
<th>Efgartigimod 10 mg/kg (N = 13)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Most common TEAEs N (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Headache</td>
<td>2 (16.7)</td>
<td>1 (7.7)</td>
<td>-</td>
</tr>
<tr>
<td>Hypertension</td>
<td>1 (8.3)</td>
<td>-</td>
<td>2 (15.4)</td>
</tr>
<tr>
<td>Vomiting</td>
<td>-</td>
<td>-</td>
<td>2 (15.4)</td>
</tr>
<tr>
<td>Cystitis</td>
<td>-</td>
<td>1 (7.7)</td>
<td>1 (7.7)</td>
</tr>
<tr>
<td>Rash</td>
<td>1 (8.3)</td>
<td>1 (7.7)</td>
<td>-</td>
</tr>
<tr>
<td>Productive cough</td>
<td>1 (8.3)</td>
<td>1 (7.7)</td>
<td>-</td>
</tr>
<tr>
<td><strong>TEAEs deemed related to study intervention N (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Headache</td>
<td>1 (8.3)</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Vomiting</td>
<td>-</td>
<td>-</td>
<td>1 (7.7)</td>
</tr>
<tr>
<td>Pubic pain</td>
<td>-</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Vaginal discharge</td>
<td>1 (8.3)</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Amenorrhoea</td>
<td>1 (8.3)</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>

argenx data; data shown from the main study
**Strong Improvement of Platelet Counts Across Doses**

- 46% of patients for both doses of efgartigimod and 58% of OLE patients realized platelet response $\geq 50 \times 10^9/L$ during at least two visits
- Novel mode of action beyond boosting platelet production or broad immune-suppression
Robust Improvement of Platelet Count

Post-hoc analysis of increasing thresholds of efficacy

<table>
<thead>
<tr>
<th>Response Rate (%)</th>
<th>placebo + SOC (N=12)</th>
<th>efgartigimod + SOC (pooled N=26)</th>
</tr>
</thead>
<tbody>
<tr>
<td>≥ 50×10⁹/L (&gt;10 cumulative days)</td>
<td>0% (N=0)</td>
<td>(N=10) 38%</td>
</tr>
<tr>
<td>≥ 100×10⁹/L</td>
<td>8% (N=1)</td>
<td>(N=11) 42%</td>
</tr>
<tr>
<td>≥ 50×10⁹/L (at least two visits)</td>
<td>25% (N=3)</td>
<td>(N=12) 46%</td>
</tr>
<tr>
<td>≥ 30×10⁹/L</td>
<td>58% (N=7)</td>
<td>(N=19) 73%</td>
</tr>
</tbody>
</table>

- Efgartigimod generated therapeutic effect at multiple relevant thresholds of efficacy
- Duration of platelets remaining ≥50×10⁹/L ranged from 1 - 20 weeks, with five patients above that platelet threshold for more than a month

Note: Increasing threshold analysis based exact logistic regression model with the baseline result as a factor
Effect Observed Across ITP Classifications and SOC

- **Case 1:** Chronic ITP (diagnosed in 1984); Low platelets at base (<10x10^9/L); eltrombopag (75 mg); 5 mg/kg efgartigimod
- **Case 2:** Persistent ITP; Low platelets at base (<10x10^9/L); watch & wait; 10 mg/kg efgartigimod
- **Case 3:** Newly diagnosed ITP, ~25x10^9/L platelets at base; Corticosteroids (12 mg) tapering to 4 mg qd in follow up; 5 mg/kg efgartigimod

Note: central lab values for the main study (until day 78). All local lab values for the extended follow-up > 78d. Include extended follow-up for case 2 and 3

Extended follow-up period shown by dotted lines, note frequency of visits dependent on medical need in this period
ITP Phase 2: Hematological Beachhead Established

- Favorable and consistent safety & tolerability profile
- Clinically meaningful & statistically significant increase of platelet count – across doses and ITP patient types
- Strong and consistent IgG reduction – validating focus on IgG mediated diseases
- Enabling Phase 3 in ITP (IV) and launch of Phase 2 in ITP for SubQ formulation
Pemphigus Vulgaris Phase 2 Adaptive Design

IDMC Recommendations in Red

Treatment Phase

Induction

3 weeks

COHORT 1: N= 4

efgartigimod (10 mg/kg)

4 infusions (weekly)

COHORT 2 & 3: N= 4 + 4

efgartigimod (10 mg/kg)

4 infusions (weekly)

Maintenance

6 weeks → 8 weeks

efgartigimod (10 mg/kg)

2 infusions (w2, w6)

4 infusions (w2, w4, w6, w8)

Follow-up Phase

8 weeks

- Cohort 1: 10 mg/kg, induction = 4 infusions (3 weeks), maintenance = 2 infusions (6 weeks)
- Additional cohorts:
  - Same dose (10 mg/kg)
  - 2 additional administrations during maintenance phase
  - Extend maintenance duration to 8 weeks
Pemphigus Vulgaris Phase 2 Interim Data

Rapid disease control in 4 out of 6 PV patients:
- 3 within 1 week
- 1 within 4 weeks

Patients with disease control:
- Mean max reduction in Pemphigus Disease Area Index (PDAI) score: 55%
- Mean max decrease in pathogenic IgGs: 57%

Favorable tolerability profile

No meaningful anti-drug antibody signals (ADA) reported
**Phase 1 Healthy Volunteer Subcutaneous Formulation**

**Open Label Trial Design**

**Inclusion criteria**
- Healthy male subjects
- 18-55 years old
- Body weight: 50 – 100 kg

**Screening/Randomization**

- Single IV dose (10 mg/kg)  
  N= 8

- Single SC dose (10 mg/kg)  
  N= 8

- 2X IV dose (20 mg/kg) + 8X SC dose (300 mg)  
  N= 8 (50-70 kg) + 8 (80-100 kg)

**Treatment Phase & Follow-up Phase**

- 3 weeks

**Read out**

- Safety & Tolerability
- PK
- PD Total IgG; IgG subtypes; IgA & IgM
- Immunogenicity

Clinicaltrials.gov: NCT03334084, argenx data
Efgartigimod: Viability of Subcutaneous Dosing

SC formulations potentially important for larger patient populations (chronic, ex-hospital)

Phase 1 Healthy Volunteer Subcutaneous Formulation

**Comparable IgG reduction**

<table>
<thead>
<tr>
<th>Days after start of administration</th>
<th>IgG % T0</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>150</td>
</tr>
<tr>
<td>20</td>
<td>100</td>
</tr>
<tr>
<td>40</td>
<td>50</td>
</tr>
<tr>
<td>60</td>
<td>0</td>
</tr>
</tbody>
</table>

- IV dose (10mg/kg)
- SC dose (10mg/kg) (N=16)

**Steady state IgG reduction (~50%)**

- IV loading
- SC maintenance
- Time (days)

- IV dose (70 kg)
- SC dose (90 kg)

- Viability of SC formulation demonstrated:
  - Comparable half-life to IV
  - Comparable IgG reduction to IV; steady state 50% IgG reduction achieved by weekly dosing (300 mg fixed dose)
  - Favorable bio-availability (~ 50%)
  - Favorable viscosity and stability profile
Efgartigimod: a Pipeline-in-a-Product Opportunity

Landscape of IgG-mediated severe autoimmune diseases (sampling)

- Immune Thrombocytopenia
  - Myasthenia Gravis
  - Multiple Sclerosis
- Scleroderma
- Lupus
- Epidermolysis Bullosa Acquisita
  - Pemphigus
  - Bullous Pemphigoid

Solid Biology Rationale
Disease proven to be predominantly mediated by pathogenic IgGs

Feasible for Biotech
Orphan potential, economically viable, efficient clinical & regulatory pathway

Proof-of Concept:
Myasthenia Gravis

Therapeutic Area Beachheads with Expansion Possibilities into Adjacent Indications

- Neuromuscular Diseases
- Hematology Disorders
- Blistering Diseases

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)
Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

What is CIDP?

- Rare, chronic autoimmune disorder of peripheral nerves, nerve roots
  - Caused by destruction of nerve myelin sheath
- Diagnosis based on clinical symptoms and electrodiagnostic findings
- US prevalence: ~16,000 patients; similar number in EU5
- IgG auto-antibodies increasingly identified in patients
- Progressive disease: symptoms include increasing loss of sensation, tingling and pain, loss of reflexes, weakness, difficulty walking, foot drop, and can lead to immobility

Limited treatment options

- IV/SC immunoglobulin *, corticosteroids
  - major IVIg indication **
- Plasma exchange
- Other immunosuppressants

Unmet need in CIDP

- Disease burden significantly underestimated
- Existing treatments are onerous and associated with significant side effects
- New treatments that are more effective and convenient, safer and better tolerated than IVIg or steroids

*Estimated IV/SC Ig US sales in CIDP reached $1B in 2014
** Robert et al, 2015

GBS/CIDP Foundation
argenx proprietary market research
ARGX-110 (Cusatuzumab): Phase 1 / 2 Mono & Combo Therapy
Cusatuzumab Mode-of-Action Targets both Leukemic Stem Cells and Blasts

Cusatuzumab induces LSC differentiation

1. Induce myeloid differentiation
2. Kill LSCs

Cusatuzumab kills blasts

3. Kill Blasts
4. Block proliferation & survival signal
   
   Activation of the pathway leads to release of sCD27, which is a biomarker

- Cusatuzumab is a potentially first-in-class anti-CD70 ADCC enhanced SIMPLE Antibody™ which selectively targets LSCs and blasts in AML and other heme indications

What is Acute Myeloid Leukemia?

- Rare hematologic cancer characterized by excessive proliferation of myeloid stem cells and their failure to properly differentiate into mature white blood cells
- AML progresses very rapidly and is fatal if left untreated
- ~22,000\(^{(1)}\) new cases per year in the U.S.
- Disease of the elderly — 60% of diagnosed patients are older than 60

Limited current treatment options

- Elderly, frail patients unfit for high dose chemotherapy — palliative treatment with hypomethylating agents
  - Median survival of 7 – 10 months
  - ~6\(^{(2)}\)% five year survival rate for patients over 65
- First-line treatments for patients <45: aggressive chemotherapy followed by stem cell transplant
  - 5-year survival is ~57\(^{(2)}\)% for patients under 45

Phase 1 – Dose Escalation

Safety and tolerability

- Vidaza = 75 mg/m² (standard of care)
- N = up to 24

Dosing Cohorts:
- 1 mg/kg, N = 3+3
- 3 mg/kg, N = 3+3
- 10 mg/kg, N = 3+3
- 20 mg/kg

Phase 2 – Proof of Concept

- Vidaza = 75 mg/m² (standard of care)
- N = 21

Dosing Cohort:
- 10 mg/kg

We are here

- Hypomethylation agents such as Azacitidine increase CD70 expression
- Population: untreated AML & high risk of myelodysplastic syndrome (MDS), eligible for AZA
- Design: open-label, non-controlled, non-randomized

Phase 1 Data from 4 Dosing Cohorts to Be Presented at ASH18

*Some Myelodysplastic Syndrome (MDS) patients are at high risk of developing AML; MDS affects bone marrow cells, reducing their ability to produce red & white blood cells

(1) Zhou et al. 2011, Lupus.
CD70 Provides Unifying Rationale Across Risk & Age Classes in AML
Potential to selectively target leukemic stem cells in AML patients

- Elevated sCD27 serum levels in all newly diagnosed AML patients, regardless of risk or age categories
- sCD27 levels are an independent negative prognostic marker in all newly diagnosed AML patients
- CD70 expressed on ~86-100% of AML blasts, majority of malignant cells are CD70/CD27 double-positive
- CD70/CD27 selectively overexpressed on leukemic stem cells (LSCs), not on hematopoietic stem cells (HSCs)

ARGX-110: Inhibits LSC Proliferation in Lasting Fashion

**Long-term effects *ex vivo***

- Reduces LSC colony formation across patient risk categories (favorable/intermediate/adverse risk)
- Reduces LSC numbers as determined in serial re-plating experiments
- Blocking CD70 results in: (1) lasting down-regulation of stem cell genes (2) increasing myeloid differentiation

ARGX-110: Curative Potential of Monotherapy in Mouse Model

Shown to reduce LSCs, increasing survival in AML model

- Increased survival after secondary transplantation of AML BM cells from primary recipients transiently treated with ARGX-110 variant
- Increased survival observed for AML blasts taken from all 3 AML risk categories (fav/int/adv)

Hypomethylating agent+ ARGX-110 Synergistically Eradicate AML LSC

**HMA-treated patients, Ex vivo colony formation assay (primary AML LSC)**

- **HMA upregulate CD70 protein and mRNA on AML LSC ex vivo (~4-fold) and in vivo (~5-7-fold) – NOT on HSC**
- **Ex vivo**: HMA/αCD70 synergistically reduce colony formation; effect maintained upon serial replating, transient treatment
- **In vivo**: Transient treatment by HMA/αCD70 eradicate human LSCs in therapeutic model; efficacy confirmed in CFU assay

**In vivo PDX model (primary AML LSC)**


- **HMA**
  - **Δ MFI CD70 (fold change)**
  - **Colonies / 10^3 CD34^+CD38^- cells**
  - **P6 fav. P8 int. P11 adv.**

<table>
<thead>
<tr>
<th></th>
<th>Veh</th>
<th>αCD70</th>
<th>D</th>
<th>αCD70/D</th>
</tr>
</thead>
<tbody>
<tr>
<td>Δ MFI</td>
<td></td>
<td></td>
<td>1</td>
<td></td>
</tr>
<tr>
<td>P6 fav.</td>
<td></td>
<td>***</td>
<td>1</td>
<td></td>
</tr>
<tr>
<td>P8 int.</td>
<td>***</td>
<td></td>
<td>1</td>
<td></td>
</tr>
<tr>
<td>P11 adv.</td>
<td>***</td>
<td></td>
<td>1</td>
<td></td>
</tr>
</tbody>
</table>

- **In vivo**
  - **human colonies / 10^5 plated BM cells**

<table>
<thead>
<tr>
<th></th>
<th>Veh</th>
<th>αCD70</th>
<th>D</th>
<th>αCD70/D</th>
</tr>
</thead>
<tbody>
<tr>
<td>P6 fav.</td>
<td>21</td>
<td></td>
<td>14</td>
<td></td>
</tr>
<tr>
<td>P8 int.</td>
<td></td>
<td>***</td>
<td>7</td>
<td></td>
</tr>
<tr>
<td>P11 adv.</td>
<td>n.s.</td>
<td></td>
<td>0</td>
<td></td>
</tr>
</tbody>
</table>

Hinterbrander ASH 2017: Blocking CD70/CD27 signaling in combination with hypomethylating agents eradicates human CD34+ AML stem and progenitor cells; manuscript in preparation
Non-Transplantable Patients with Intermediate & Adverse Risk and High Blast Count in Bone Marrow

9 newly diagnosed AML patients

<table>
<thead>
<tr>
<th>Baseline characteristics (N=9)</th>
<th>ARGX-110 + Azacitidine</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>1 mg/kg</td>
</tr>
<tr>
<td><strong>Age</strong></td>
<td></td>
</tr>
<tr>
<td>Median</td>
<td>71</td>
</tr>
<tr>
<td>71-80</td>
<td></td>
</tr>
<tr>
<td>Gender: Male/Female</td>
<td>2/1</td>
</tr>
<tr>
<td>Risk (ELN 2017)</td>
<td></td>
</tr>
<tr>
<td>Intermediate</td>
<td>1</td>
</tr>
<tr>
<td>Adverse</td>
<td>2</td>
</tr>
<tr>
<td>Blasts in the bone marrow</td>
<td></td>
</tr>
<tr>
<td>Median %</td>
<td>51.3</td>
</tr>
<tr>
<td>24-90</td>
<td></td>
</tr>
<tr>
<td>AML classification (WHO 2016)</td>
<td></td>
</tr>
<tr>
<td>Not other specified</td>
<td></td>
</tr>
<tr>
<td>With Myelodysplasia- related changes</td>
<td>2</td>
</tr>
<tr>
<td>Therapy-related myeloid neoplasm</td>
<td>1</td>
</tr>
<tr>
<td>French-American-British subtypes</td>
<td>M4,M1,M2</td>
</tr>
</tbody>
</table>

ELN: European Leukemia Net, Dohner et al. 2017, Blood

Source: argenx data – patient anecdotes – uncleaned data
Response in 6/6 Evaluable Newly Diagnosed AML Patients
ARGX-110/Aza treatment

- So far, all patients responded (3 CR, 1 CRi, 2 PR)
- 1 patient reached CR and bridged to allogeneic stem cell transplant after 5 cycles
- 6/9 patients were still on treatment

Case Study: Cusatuzumab/Aza Induces Complete Remission and Bridges to Transplant

- 75 year old male; AML with myelodysplasia-related changes, M1; BM 40% blasts CM
- Molecular genetics: U2AF1mut; DNMT3Amut; cytogenetics: normal

Screening

Leukemic blast persistence – (C1D1)

Leukemic clearance – (C4D1)

Bone marrow: % Blasts, flow cytometry

Blood analysis: Absolute counts (G/L)

MRD negative
Case Study: Cusatuzumab/Aza Combo Reduces AML Stemness

- Significantly reduced LSC colony formation
- Increased myeloid differentiation (asymmetric division) of LSCs
- Reduction of LSC gene signature

Ng et al. 2016, Nature
ARGX-110 in Newly Diagnosed AML Patients

Ongoing Phase 1 dose-escalation part of the Phase 1/2 clinical trial (12 patients)

Data cut-off of July 16 - Abstract published for ASH

**Encouraging proof of biology data** in 12 patients (4 dose cohorts; 3 patients each)
- 11/12 responders
  - Complete response: 8 patients (73%)
  - Complete response without hematologic recovery: 1 patient (9%)
  - Morphologic leukemia-free status: 1 patient (9%)
  - Partial response: 1 (9%)
- Minimal residual disease (MRD) negativity: 5/12 (42%)
- Median response duration: 6.9 months (up to 14.4 months – analysis still ongoing)
- ARGX-110 monotherapy & in combo with AZA reduce leukemic stem cells in BM

**Encouraging safety** and tolerability profile
- No exacerbation of azacitidine toxicity observed
- No dose limiting toxicity
- Safety profile enabling combination therapy (with ao hypomethylating agents)

**Highly differentiated drug profile**
- CD70 uniformly & selectively expressed
- Driving LSCs into myeloid differentiation
Business development & financials
AbbVie Partnership for Novel Target GARP

**Strategic Antibody Collaboration Details**

- **GARP** is a protein specifically present on the surface of activated regulatory T-cells (Tregs)
- **AbbVie** exercised option in August 2018 to:
  - Obtain exclusive, worldwide license to develop and commercialize ARGX-115
  - Fund further GARP-related research by argenx beyond ARGX-115
  - **argenx** can study ARGX-115 in combo with its pipeline programs

**Financial Highlights**

- $60mm received to date
- $625mm in potential development, regulatory and commercial milestones
- **Tiered royalties** on sales at percentages ranging from mid-single digits to low teens
- **Co-promotional** rights for ARGX-115-based products in the European Economic Area and Switzerland
## Additional Strategic Collaborations

<table>
<thead>
<tr>
<th>Partner</th>
<th>Asset</th>
<th>Key commentary</th>
</tr>
</thead>
</table>
| Genor Biopharma | ARGX-109 (Gerilimzumab) | - Anti-IL-6 antibody for rheumatoid arthritis (P1 concluded)  
- Development for Chinese market |
| LEO | ARGX-112 | - Focused on **inflammation-based dermatological indications**  
- LEO Pharma funds >50% of all development costs up to CTA approval and all development post-approval of first Phase 1 trial in Europe  
- argenx is eligible for ~€100mm in aggregate milestone payments + tiered royalties |
| STATEN BIOTECHNOLOGY | ARGX-116 | - Focused on **dyslipidemia**  
- Jointly responsible for conducting dyslipidemia research — Staten responsible for additional clinical development  
- argenx eligible for royalties in the low twenties |
| Shire | | - Focused on **novel rare disease targets**  
- Provides Shire access to SIMPLE Antibody™ platform + Fc engineering technologies  
- argenx has received $12.6 mm in aggregate upfront and milestone payments and R&D fees over the course of the collaboration  
- Shire purchased €12mm of argenx ordinary shares through participation in July 2014 IPO |
Financial Profile and Investor Composition

Shareholder base > 70% U.S. investors

**Additional Key Statistics – Sept 30, 2018**

- Cash position: €582.3 mm
- Capital raised since inception: €730 mm (ex. grants)
  - 2017: raised $115 mm (€102 mm) in Nasdaq IPO
  - 2017: raised $266 mm (€226 mm) in public offering
  - 2018: raised $300 mm (€256 mm) in public offering
- Non-dilutive funding since inception: €104 mm (incl. grants)
  - 2018: $10 mm second preclinical milestone AbbVie
- 120 employees & consultants — 89 R&D, 31 SG&A

**Blue-Chip Investor Base – Sept 30, 2018**

- U.S. shareholding expanded *above* 70%
- Outstanding shares: 35,934,457
Appendix
Augmenting Intrinsic Therapeutic Properties of Antibodies

Leadership in discovery and application of novel biology

- Extends half-life / PD effect
- Enhances tissue penetration
- Clears disease target
- Clears autoantibodies
- Boosts cell killing

**V-region**

- Unlock novel and complex targets

**Fc region**

- Modulate immune response

**SIMPLE Antibody™ Platform**
- Llama immune system delivers V-regions with high human homology
- Highly diverse antibody output covers a multitude of target epitopes

**NHance®**
- Extends half-life / PD effect
- Enhances tissue penetration

**ABDEG™**
- Clears disease target
- Clears autoantibodies

**POTELLIGENT®**
- Boosts cell killing

Unique suite of technologies enables development of differentiated product candidates against novel targets

Klarenbeek et al. 2015, mAbs
Basilico et al. 2014, J Clin Inv
Please Join argenx for a Lunch and Discussion during the ASH Annual Meeting

Proof-of-Concept of Efgartigimod (ARGX-113) in Immune Thrombocytopenia (ITP)
Advancing Cusatuzumab (ARGX-110) in Acute Myeloid Leukemia (AML)

**Monday, December 3, 2018**
12:00 – 1:30 PM PT
Event will be webcast

**Hilton San Diego Bayfront**
Aqua 310
1 Park Blvd
San Diego, CA 92101
Two minute walk from Convention Center

**Agenda**
- Full Data from Phase 2 Clinical Trial of Efgartigimod in ITP
- Phase 1 Dose-Escalation Trial of Cusatuzumab in AML

**Guest Speaker**
**Dr. Adrian C. Newland**
Barts and the London School of Medicine and Dentistry
The Royal London Hospital

**Dr. Adrian F. Ochsenbein**
Department of Medical Oncology
University of Bern
Bern University Hospital