AUAT-201 Data

Phase 2 randomized, double-blind, parallel-group, placebo-controlled trial, evaluated the safety, efficacy, and dose response of three doses of ATI-501 on the regrowth of hair in subjects with Alopecia Areata (AA).

JULY 2019
AUAT-201: Study Design

Randomized, Double-blind, Placebo-controlled Multicenter Study

Eligibility

• AA Diagnosis
  • Stable AA, Alopecia Universalis or Alopecia Totalis
• 30-100% total scalp hair loss (SALT)
• 6 months -12 years in duration of current episode of scalp hair loss

1:1:1:1

Randomize

N=87

Treatment – 24 weeks

Endpoints

Safety & Tolerability

Primary Efficacy:
• % change in SALT at Week 24

Secondary Efficacy includes:
• Absolute change in SALT scores at Week 24
• Alopecia Density and Extent (ALODEX) percent change at Week 24
• ALODEX absolute change at Week 24

Assessment
(Every 4 Weeks, Weeks 4 through 28)

ATI-501, 800 mg, BID
ATI-501, 600 mg, BID
ATI-501, 400 mg, BID
placebo, BID

25 US clinical sites
ClinicalTrials.gov ID NCT03594227

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Subject Disposition

Enrolled
N=136

Randomized
N=87

Allocation
- Pbo BID N=19
- 400mg BID N=23
- 600mg BID N=23
- 800mg BID N=22

Completed (PP) = 70
- N=14 (74%)
- N=18 (78%)
- N=19 (83%)
- N=19 (86%)

Discontinued Reasons
- N=5 (26%)
  - AE 2 (11%)
  - Withdrew Consent 1 (5%)
  - Other 2 (11%)
- N=5 (22%)
  - AE 2 (9%)
  - Loss to Follow-Up 1 (4%)
  - Withdrew Consent 1 (4%)
  - Other 1 (4%)
- N=4 (17%)
  - AE 1 (4%)
  - Loss to Follow-Up 1 (4%)
  - Withdrew Consent 1 (4%)
  - Pregnancy 1 (4%)
- N=3 (14%)
  - Loss to Follow-Up 1 (5%)
  - Withdrew Consent 1 (5%)
  - Other 1 (5%)
### Demographics & Baseline Characteristics

<table>
<thead>
<tr>
<th></th>
<th>Pbo, BID N=19</th>
<th>400 mg, BID N=23</th>
<th>600 mg, BID N=23</th>
<th>800 mg, BID N=22</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Age Mean (SD)</strong></td>
<td>41.8 (16.01)</td>
<td>38.7 (12.99)</td>
<td>40.4 (13.56)</td>
<td>40.5 (12.44)</td>
</tr>
<tr>
<td><strong>Sex Male N(%)</strong></td>
<td>5 (26.3%)</td>
<td>6 (26.1%)</td>
<td>11 (47.8%)</td>
<td>9 (40.9%)</td>
</tr>
<tr>
<td><strong>Sex Female N(%)</strong></td>
<td>14 (73.7%)</td>
<td>17 (73.9%)</td>
<td>12 (52.2%)</td>
<td>13 (59.1%)</td>
</tr>
<tr>
<td><strong>Race</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>White</td>
<td>15 (78.9%)</td>
<td>17 (73.9%)</td>
<td>17 (73.9%)</td>
<td>14 (63.6%)</td>
</tr>
<tr>
<td>African American</td>
<td>3 (15.8%)</td>
<td>6 (26.1%)</td>
<td>3 (13.0%)</td>
<td>5 (22.7%)</td>
</tr>
<tr>
<td>Other</td>
<td>1 (5.3%)</td>
<td>0</td>
<td>3 (13.0%)</td>
<td>3 (13.6%)</td>
</tr>
<tr>
<td><strong>Diagnosis</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Areata</td>
<td>9 (47.4%)</td>
<td>11 (47.8%)</td>
<td>14 (60.9%)</td>
<td>9 (40.9%)</td>
</tr>
<tr>
<td>Universalis</td>
<td>5 (26.3%)</td>
<td>4 (17.4%)</td>
<td>5 (21.7%)</td>
<td>7 (31.8%)</td>
</tr>
<tr>
<td>Totalis</td>
<td>5 (26.3%)</td>
<td>8 (34.8%)</td>
<td>4 (17.4%)</td>
<td>6 (27.3%)</td>
</tr>
<tr>
<td><strong>Mean Duration of Alopecia (yrs, SD)</strong></td>
<td>11.3 (12.04)</td>
<td>13.7 (11.01)</td>
<td>9.5 (9.78)</td>
<td>10.7 (11.36)</td>
</tr>
<tr>
<td><strong>Mean Duration of Current Alopecia episode (yrs, SD)</strong></td>
<td>4.9 (2.94)</td>
<td>4.3 (3.54)</td>
<td>3.6 (2.84)</td>
<td>3.4 (3.11)</td>
</tr>
<tr>
<td><strong>Mean Baseline SALT (SD)</strong></td>
<td>85 (24.9)</td>
<td>78 (26.7)</td>
<td>76 (23.4)</td>
<td>81 (25.8)</td>
</tr>
</tbody>
</table>
Primary Endpoint

Percent Change From Baseline in SALT Scores Over Time (ITT Population)

Model Based Means (95% CI)

Week

Treatment
- Placebo
- 400 mg
- 600 mg
- 800 mg

400 mg vs Placebo; Diff = -19, P-Value = 0.011 *
600 mg vs Placebo; Diff = -24, P-Value = 0.001 *
800 mg vs Placebo; Diff = -20, P-Value = 0.010 *
Secondary Endpoint

Percent Change From Baseline in ALODEX Scores Over Time (ITT Population)

- Model Based Means (95% CI)
- Treatment:
  - Placebo
  - 400 mg
  - 600 mg
  - 800 mg

Week:
- 5
- 10
- 15
- 20
- 25

400 mg vs Placebo; Diff = -19, P-Value = 0.013 *
600 mg vs Placebo; Diff = -25, P-Value = 0.002 *
800 mg vs Placebo; Diff = -19, P-Value = 0.014 *
Secondary Endpoint

Absolute Change From Baseline in SALT Scores Over Time (ITT Population)

Model Based Means (95% CI)

Treatment
- Placebo
- 400 mg
- 600 mg
- 800 mg

Week

400 mg vs Placebo; Diff = -11, P-Value = 0.025 *
600 mg vs Placebo; Diff = -15, P-Value = 0.003 *
800 mg vs Placebo; Diff = -18, P-Value < 0.001 *
Secondary Endpoint

Absolute Change From Baseline in SALT Scores Over Time
(Baseline SALT Group >= 50, ITT Population)

Model Based Means (95% CI)

- Placebo
- 400 mg
- 600 mg
- 800 mg

400 mg vs Placebo; Diff = -11, P-Value = 0.064
600 mg vs Placebo; Diff = -15, P-Value = 0.008 *
800 mg vs Placebo; Diff = -23, P-Value < 0.001 *

Week
Secondary Endpoint

Proportion of Subject Achieving SALT50 Over Time (Baseline SALT >= 50 Subgroup)

- 400 mg vs Placebo; Odds Ratio = 7.5, P-Value = 0.209
- 600 mg vs Placebo; Odds Ratio = 11.0, P-Value = 0.125
- 800 mg vs Placebo; Odds Ratio = 14.5, P-Value = 0.088

Model Based Means (95% CI)

Treatment
- Placebo
- 400 mg
- 600 mg
- 800 mg

Week
# Overall Summary of Adverse Events (AEs)

<table>
<thead>
<tr>
<th></th>
<th>Placebo oral suspension (N=19)</th>
<th>ATI-501 oral suspension 400 mg BID (N=23)</th>
<th>ATI-501 oral suspension 600 mg BID (N=23)</th>
<th>ATI-501 oral suspension 800 mg BID (N=22)</th>
<th>All ATI-501 subjects (N=68)</th>
<th>All subjects (N=87)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Subjects with at least one AE</td>
<td>14 (73.7%)</td>
<td>16 (69.6%)</td>
<td>16 (69.6%)</td>
<td>15 (68.2%)</td>
<td>47 (69.1%)</td>
<td>61 (70.1%)</td>
</tr>
<tr>
<td>Subjects with at least one SAE</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Subjects with at least one severe AE</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Subjects with at least one related AE</td>
<td>3 (15.8%)</td>
<td>5 (21.7%)</td>
<td>2 (8.7%)</td>
<td>3 (13.6%)</td>
<td>10 (14.7%)</td>
<td>13 (14.9%)</td>
</tr>
<tr>
<td>Subjects with at least one AE leading to discontinuation of study drug</td>
<td>2 (10.5%)</td>
<td>2 (8.7%)</td>
<td>1 (4.3%)</td>
<td>0</td>
<td>3 (4.4%)</td>
<td>5 (5.7%)</td>
</tr>
<tr>
<td>Subjects with at least one related AE leading to discontinuation of study drug</td>
<td>1 (5.3%)</td>
<td>1 (4.3%)</td>
<td>0</td>
<td>0</td>
<td>1 (1.5%)</td>
<td>2 (2.3%)</td>
</tr>
</tbody>
</table>
- Male 35 yrs
- 400mg BID
- AA Disease = 1.0 yrs; Current Episode 1.0 yrs.
- SALT 54% to 2%
- Female 48 yrs
- 600mg BID
- AA Disease = 38.7 yrs; Current Episode 1.1 yrs
- SALT 100% to 0%.
• Male 53 yrs
• 800mg BID
• AA Disease = 23.6 yrs; Current Episode 4.6 yrs.
• SALT 100% to 17%.
Summary

• Subjects in each of the three ATI-501 active dose groups (400 mg, 600 mg and 800 mg) had statistically significant improvements compared to placebo for the primary endpoint (p=0.011, p=0.001 and p=0.010, respectively).

• ATI-501 was generally well-tolerated at all doses.
  ✓ There were no serious adverse events
  ✓ All adverse events AEs) were mild or moderate in severity
  ✓ No thromboembolic events observed
  ✓ The most common AEs across all groups were: nasopharyngitis, influenza, upper respiratory tract infection, urinary tract infection, acne, blood creatine phosphokinase increased, and sinusitis
THANK YOU