First-in-human, First-in-class Phase 1a Study of BXQ-350 for Solid Tumors and Gliomas.

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**Background:** BXQ-350 is a novel agent composed of the multifunctional, lysosomal activator protein Saposin C and phosphatidylserine. BXQ-350 demonstrated antitumor effects *in vitro* and *in vivo*, particularly in glioma and pancreatic cancer models.

**Methods:** A phase 1a dose-escalation trial (NCT02859857; Phase 1b ongoing) was conducted in refractory solid tumors/high-grade glioma (HGG) patients (Pt) by administering escalating IV BXQ-350 doses of 0.7, 1.1, 1.4, 1.8, or 2.4 mg/kg on days 1-5, 8, 10, 12, 15, 22 (cycle 1) and at 28-day cycles thereafter. Response was assessed at day 113 by RECIST or RANO.

**Results:** 17 Pt (age 24-67) with a median 7 (range, 2-12) prior systemic therapies completed a median 2 (range, 1-6) cycles without DLTs or treatment-related serious adverse events (AEs). Moderately severe related AEs occurred in 3 (100%), 1 (33%), 1 (33%), and 2 (25%) Pt at 1.1, 1.4, 1.8, and 2.4 mg/kg cohort doses, respectively. The most common treatment-related AEs was transient fatigue (n = 4, 23.5%). At 2.4 mg/kg, 1 Pt had moderate blood pressure elevation. Best response in 7 Pt completing to day 113 was 1 PR (appendiceal carcinoma) at 2.4 mg/kg, and 6 SD (1 HGG Pt at 0.7 mg/kg had stable disease >12+ months, and 6 had improved day 113 RANO/RECIST). BXQ-350 pharmacokinetics was dose proportional with half-life and Cmax, consistent with preclinical data.

**Conclusions:** BXQ-350 showed clinical activity in heavily pre-treated patients with advanced solid and brain tumors. BXQ-350 has a tolerable safety profile with no significant DLT at the highest planned dose, supporting continued monotherapy dose expansion at 2.4 mg/kg.

<table>
<thead>
<tr>
<th>DOSE (mg/kg)</th>
<th>0.7</th>
<th>1.1</th>
<th>1.4</th>
<th>1.8</th>
<th>2.4</th>
</tr>
</thead>
<tbody>
<tr>
<td>N</td>
<td>1</td>
<td>3</td>
<td>3</td>
<td>3</td>
<td>8</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Mean Age, F:M</th>
<th>64, 0:1</th>
<th>53, 0:3</th>
<th>58, 2:1</th>
<th>49, 1:2</th>
<th>54, 2.2</th>
</tr>
</thead>
<tbody>
<tr>
<td>Prior systemic therapy, # cycles, range</td>
<td>7, 6</td>
<td>5-7, 2-4</td>
<td>2-12, 1-3</td>
<td>3-8, 2-6</td>
<td>4-12, 1-4</td>
</tr>
<tr>
<td>Solid Tumor n, Improved RANO n/N D 113</td>
<td>1, 0</td>
<td>2, 1/1</td>
<td>1, 1/2</td>
<td>1, 0</td>
<td>5, 0</td>
</tr>
</tbody>
</table>
HGG n, Improved RECIST, n/N D 113 1, 1/1 1, 0 2, 0 2, 0 3, 2/2

Adverse Event (n case, n event) 1, 15 3, 54 3, 37 3, 32 8, 80

Moderate severity related 3, 6 1, 1 1, 2 2, 2

Fatigue 2, 2 1, 2 1, 1

Neutropenia, EKG, Balance, Nerv, Dysarthria, Urin, BP 3, 4

Serious non-related – GI, hyponatremia, weak 1, 1 1, 4 1, 3 1, 3 3, 5

Title:
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No

Is this abstract a clinical trial?
Yes

Is this clinical trial registered?
Yes

Registry Name:
Clinicaltrials.gov

Registration Number:
NCT 02859857

Research Funding Source:
Pharmaceutical/Biotech Company

Research Funding Source Name:
Bexion Pharmaceutical

Would like to be considered for a Merit Award:
No

Have the data in this abstract been presented at another major medical meeting?
No

Has this research been submitted for publication in a medical journal?
No

Type of Research:
Phase I

Research Category:
Clinical

Continued Trial Accrual:
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