



## **TopiVert commences Phase I study with oral TOP1288 in the treatment of ulcerative colitis**

**London, UK, 28 February 2017: TopiVert Pharma Ltd** (“TopiVert” or the “Company”), a clinical-stage biotechnology company developing Narrow Spectrum Kinase Inhibitors (NSKI) as novel, locally-acting medicines for the treatment of chronic inflammatory gastrointestinal and ocular diseases, today announces the successful dosing of the first subjects in a Phase 1 study of its oral formulation of TOP1288 for the treatment of ulcerative colitis (UC).

UC is a form of inflammatory bowel disease (IBD) affecting the colon. As many as 40% of patients fail to respond to current drug therapy, which is often poorly tolerated, and approximately 20% of patients require surgical removal of the colon to manage the disease. TOP1288 is an NSKI developed by TopiVert that targets several important kinases in the signalling cascade in inflammatory cells, leading to synergistic inhibition of key pathways involved in innate and adaptive immunities. Furthermore, TOP1288 has very low systemic bioavailability such that its activity is confined to the site of active disease in UC patients and providing an improved safety and tolerability profile.

The Phase 1 study is designed to assess the safety, pharmacokinetics and pharmacodynamics of TOP1288 when delivered orally in healthy volunteers. A Phase 2a proof of concept study of a rectal formulation of TOP1288, which is ongoing in Europe, aims to demonstrate the efficacy and safety of the compound in the treatment of UC when the drug is delivered directly to the site of disease in the colon. This POC study with a rectal formulation is intended to guide the development of an oral formulation of TOP1288 as the intended commercial presentation. Results from both oral and rectal studies are expected in the second half of 2017.

**Ajay Duggal, TopiVert’s Chief Medical Officer, commented:** *“We are delighted to have commenced the clinical evaluation of an oral formulation of TOP1288, as our intended commercial presentation. The Phase I study will evaluate the ability to deliver TOP1288 to the colon by the oral route, as well as assessing safety and tolerability of the formulation. In parallel, a rectal formulation of TOP1288 is being evaluated in a Phase 2a study to demonstrate proof of concept in UC patients. Both studies will report in the second half of this year. Given the pressing need for improved treatments in UC, we are eager to see if TOP1288’s potent anti-inflammatory properties can bring clinical benefit to UC patients.”*

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### **For more information, please contact:**

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### **About TopiVert**

TopiVert is a clinical-stage biotechnology company developing narrow spectrum kinase inhibitors as novel, locally-acting medicines for the treatment of chronic inflammatory gastrointestinal (GI) and ocular diseases. The Company's lead GI programme, TOP1288 for the treatment of ulcerative colitis, is currently being evaluated in two clinical studies: a Phase 2a proof of concept study with a rectal formulation, and a Phase 1 study with an oral formulation of TOP1288. In parallel, its lead ophthalmology programme, TOP1630 for dry eye syndrome (DES), is currently being evaluated in a Phase 1/2a proof of concept study in the US. All three clinical studies are due to report in the second half of 2017. Current therapies for these debilitating diseases provide inadequate long-term control in a high proportion of patients and considerable unmet medical need remains. The Company commenced operations in early 2012 and its investors include Imperial Innovations, SV Life Sciences, NeoMed and Johnson & Johnson Innovation-JJDC, Inc.

### **About Narrow Spectrum Kinase Inhibitors (NSKIs)**

NSKIs are novel small molecules characterised by broad, potent anti-inflammatory activity and minimal systemic exposure. Specifically, NSKIs are potent inhibitors of a select range of pivotal kinases involved in inflammatory cascades of both innate and adaptive immunities. Simultaneous targeting of multiple inflammatory components leads to a synergistic activity profile with broad anti-inflammatory effects. The NSKIs are designed to have low systemic bioavailability so that their exposure to the body's healthy tissues is reduced and thus their safety and tolerability enhanced. Together, these attributes make NSKIs ideal treatment candidates for chronic inflammatory diseases where long term therapy demands a sustained effect accompanied by excellent safety and tolerability.

### **About IBD and ulcerative colitis**

Inflammatory bowel disease (IBD) is a term used to describe several diseases that involve inflammation of the gastrointestinal tract. The two most common forms of IBD, Crohn's disease and ulcerative colitis, together affect over 4 million people worldwide. They are both chronic relapsing conditions that cause bloody diarrhoea, abdominal pain and significant reductions in a patient's quality of life. While their causes are not fully understood, these diseases are characterised by an abnormal inflammatory reaction that leads to damage of the intestinal wall.

Current treatments for ulcerative colitis involve administration of oral, rectal or intravenous/subcutaneous anti-inflammatory and immunomodulatory therapies, including biologics. Despite these products being effective in treating active disease in some patients, their long term use is often hampered by safety and tolerability issues. Furthermore, at least 40% of patients have poorly controlled disease and around 20% require surgery to manage the disease.