Elpamotide

Also called: VEGFR2-169; VEGFR2-169 peptide vaccine; OTS-102; OTS102; Elpamotida; RFVPDGNRI

What it is

Elpamotide (VEGFR2-169, OTS-102; RFVPDGNRI) is an HLA-A*24:02-restricted epitope peptide from VEGFR2, given as a weekly injection to raise cytotoxic T cells against tumour vessel lining. It induced peptide-specific T cells in most recipients (PMIDs 19930156, 22842485), but adding it to gemcitabine did not prolong survival in the randomised phase 2/3 PEGASUS-PC trial of 153 patients with advanced pancreatic cancer (PMID 25867139). It is approved nowhere.

Approval status

Not FDA Approved. Never approved: Drugs@FDA and the openFDA label endpoint hold no record for elpamotide (searched November 4, 2026), and ChEMBL records a highest clinical phase of 2 (CHEMBL2108295). The programme ran in Japan: a phase 1 trial with gemcitabine (NCT00622622), a phase 1 trial in solid tumours (UMIN000008336), the randomised phase 2/3 PEGASUS-PC trial (UMIN000001664), which missed its overall-survival endpoint, and a single-arm phase 2 trial in biliary tract cancer (UMIN000002500). Two further ClinicalTrials.gov records pair it with a VEGFR1 peptide (NCT00655785, NCT00639925); none of the three posted results.

Dosing, as printed in the page's Recommended Dosing box

Used in human trials

No manufacturer dose exists for this use: these are amounts given in published trials, not recommendations.

Protocol and source	Dose	Frequency	Duration	Route
Study: PEGASUS-PC phase 2/3, pancreatic cancer **[Human study]** PMID 25867139	2 mg	Once a week	Until the disease progressed	Subcutaneous, as a water-in-oil emulsion with Montanide ISA51 VG
Study: phase 2, biliary tract cancer **[Human study]** PMID 25502982	2 mg	Once a week	Four injections per four-week course	Subcutaneous, with weekly gemcitabine
Study: phase 1 dose escalation with gemcitabine **[Human study]** PMID 19930156	0.5, 1 or 2 mg	Once a week	28-day cycles, six patients per dose	Subcutaneous
Study: phase 1 in advanced solid tumours **[Human study]** PMID 22957712	0.5, 1 or 2 mg	Once a week	28-day cycles	Subcutaneous, peptide alone

Educational information, not medical advice. Each dose above is tagged with its source: an approved label, a published human study, animal data, or amounts reported by users. None is a recommendation for you. An unapproved compound has no established safe or effective human dose, and products sold for "research use only" are not made or tested for people. Talk to a doctor before acting on anything on this site, including before you start, stop or change any medicine or dose.

Key human evidence

The first 3 of the 6 human studies carded on the page (6 study cards in all):

• **Phase 1 with gemcitabine in advanced pancreatic cancer** (Human, 2010). Patients with metastatic or unresectable pancreatic cancer received gemcitabine at 1,000 mg/m2 on days 1, 8 and 15 of a 28-day cycle, plus a weekly injection of the peptide at 0.5, 1 or 2 mg in cohorts of six. Among the 18 who completed at least one course, no adverse event of grade 4 or worse was attributed to the treatment, 15 (83%) reacted at the injection site, and peptide-specific cytotoxic T cells appeared in 11 (61%). Two-thirds had disease control and median overall survival was 8.7 months. The authors judged 2 mg or more the dose to carry forward on immunological grounds. Registered as NCT00622622. **PMID 19930156**

• **The same phase 1 trial reported in Japanese, with the dose rationale** (Human, 2011). A Japanese-language account of the pancreatic phase 1 trial from the same group, written as the randomised trial was being launched. It sets out why VEGFR2 was chosen as a target: the receptor is abundant on newly formed tumour vessels and largely absent from normal ones, and attacking the vessels rather than the tumour cells avoids the escape routes cancer cells use to hide from immune attack. It repeats the trial's findings, gives 2 mg as the dose the immunology favoured, and records that the randomised phase 2/3 trial opened in January 2009. No new patient data. **PMID 22202233**

• **Phase 1 in advanced solid tumours: no dose limit reached** (Human, 2012). Ten patients with refractory solid tumours, all positive for HLA-A*24:02, received the peptide alone under the skin weekly at 0.5, 1 or 2 mg on a 28-day cycle. No grade 3 or worse event occurred and no maximum tolerated dose was found. Five patients had grade 1 or 2 injection-site reactions, the commonest event; at the 1 mg level one patient had grade 1 proteinuria and another grade 1 proteinuria with raised blood pressure, the kind of effect expected when VEGF signalling is interfered with. Soluble VEGFR2 in blood fell significantly after vaccination, and gene-expression analysis of blood cells showed changes in T-cell and angiogenesis pathways, which the authors read as evidence the vaccine was biologically active. Registered as UMIN000008336. **PMID 22957712**

Interactions

A PubMed search for interaction studies of this compound with any drug (November 4, 2026, 0 records) found none in people. That is not evidence that it is safe to combine with anything.

Side effects and cautions

Reported side effects	Contraindications and cautions
• Reactions where the injection went in, in 83% of patients in the pancreatic phase 1 trial (PMID 19930156) • Injection-site ulceration or erosion in a minority of vaccinated patients in the randomised trial, and in no one on placebo (PMID 25867139) • Grade 1 or 2 injection-site reactions as the commonest event in the solid-tumour phase 1 trial, with no grade 3 or worse event at any dose (PMID 22957712) • Grade 1 protein in the urine, in one case with raised blood pressure, at the 1 mg dose level: the effects expected when VEGF signalling is disturbed (PMID 22957712) • More low-grade fever and raised liver enzymes than placebo in the randomised trial, on a background of gemcitabine toxicity that dominated both arms (PMID 25867139) (1 more on the page)	• Not an available medicine: it is approved nowhere, and no trial of it is recruiting • Useless without the matching tissue type: without HLA-A*24:02 there is nothing to present the peptide to T cells (PMID 22957712) • No benefit on the one endpoint that was tested properly: overall survival in a placebo-controlled trial of 153 patients (PMID 25867139) • Interferes with a pathway the body needs: the phase 1 trial saw the proteinuria and blood-pressure signal familiar from other drugs against VEGF signalling (PMID 22957712) • Studied only in adults with advanced cancer, always with chemotherapy except in one small phase 1 trial

Five questions to ask

1. Elpamotide is listed here as “Not FDA Approved”. What does the evidence on this sheet mean for someone in my situation, and is there an approved treatment I should consider instead?
2. Here are the medicines and supplements I take. Do any of them interact with Elpamotide? This sheet records none published, which does not mean none exist.
3. Which side effects should make me stop and call you, for example reactions where the injection went in?
4. Do any of the cautions on this sheet apply to me, such as: Not an available medicine: it is approved nowhere, and no trial of it is recruiting?
5. What should we check before I start and while I am on it (blood tests, blood pressure, weight), and how often?

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