UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

September 30, 2019
Date of Report (Date of earliest event reported)

X4 Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**001-38295**	**27-3181608**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

955 Massachusetts Avenue, 4th Floor **Cambridge, Massachusetts** **(Address of principal executive offices)**	**02139** **(Zip Code)**

Registrant's telephone number, including area code: (857) 529-8300

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	**XFOR**	**The Nasdaq Capital Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Item 7.01 Regulation FD Disclosure

On September 30, 2019, X4 Pharmaceuticals, Inc. (the "Company") issued a press release announcing data from the Phase 2a portion of its open-label Phase 1/2 clinical trial of its lead product candidate, mavorixafor (X4P-001), in combination with axitinib in patients with advanced clear cell renal cell carcinoma ("ccRCC"). The Company will host a conference call and webcast to discuss the results at 8:00 AM EDT on September 30, 2019. A live audio webcast of the presentation will be available under "Events and Presentations" in the "Investors" section of the Company's website at www.x4pharma.com. Alternatively, callers may listen to the conference call by phone by dialing (866) 721-7655 (U.S.) or (409) 216-0009. The conference ID number is 4787329. The webcast will be archived on the Company's website for at least 30 days. The information contained in, or that can be accessed through, the Company's website is not a part of this filing.

A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference. The information furnished under this Item 7.01, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or subject to the liabilities of that section. The information shall not be deemed incorporated by reference into any other filing with the Securities and Exchange Commission made by the Company, regardless of any general incorporation language in such filing.

Item 8.01 Other Events.

On September 30, 2019, the Company announced data from the Phase 2a portion of its open-label Phase 1/2 clinical trial of its lead product candidate, mavorixafor (X4P-001), in combination with atixinib in patients with advanced ccRCC.

Combination therapy with mavorixafor and approved tyrosine kinase inhibitor ("TKI") axitinib was observed to generally be well tolerated in the trial, with a manageable safety profile and also demonstrated clinical improvement with encouraging median progression free survival ("mPFS") in a heavily pretreated advanced ccRCC patient population. Of the 65 patients in the trial, 49 patients (75%) received mavorixafor + axitinib as a third- to ninth-line therapy, having previously been treated prior to the enrollment in the trial with between two and eight prior therapies with a TKI, immuno-oncology ("IO") agent, or other systemic therapy. Fifty-seven of the 65 patients in the trial (88%) had an intermediate or poor prognosis.

Overall mPFS in the trial across clinically evaluable patients receiving mavorixafor + axitinib (n=62) was 7.4 months. Predefined subpopulations examined patients with immediate prior TKI and IO treatment. Patients treated in the subgroup with immediate prior TKI therapy (n=34) demonstrated an objective response rate (ORR) of 18% and an increased mPFS of 7.4 months. Patients treated with mavorixafor + axitinib in the subgroup with immediate prior IO therapy (n=18) had an ORR of 61% and an increased mPFS of 11.6 months. In addition, eight of the 65 patients remain on the combination therapy as of [September 30, 2019], with durations of treatment of 17 months or longer.

This Phase 1/2, multi-center, open-label trial of mavorixafor in combination with axitinib included 65 patients with histologically confirmed advanced ccRCC, all of whom received at least one prior systemic therapy prior to enrollment in the trial. The safety analyses included 65 patients from Phases 1/2 of the trial who were treated with 400 mg mavorixafor (200 mg twice daily or 400 mg once daily) + 5 mg axitinib twice daily. Treatment responses were assessed using Response Evaluation Criteria in Solid Tumor, or RECIST v1.1 (a validated set of criteria to assess changes in tumor burden), every eight weeks from day one for 80 weeks, and then every 12 weeks thereafter, by blinded, independent central review. Treatment-related serious adverse events were diarrhea, hyperkalemia and hypertension (n=2, or 3%) and blood creatinine increased, dehydration, fatigue, hepatic enzyme increase, nausea, sepsis, trachea-oesophageal fistula, and vomiting (n=1 each, or 1.5%).

Additionally, as noted above under Item 7.01, members of the management team of the Company will be holding a conference call and live webcast to discuss the data from the clinical trial. A copy of the slide presentation to be used by the Company during the conference call is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

Exhibit No.	Description
99.1	Press Release dated September 30, 2019.
99.2	Conference Call Presentation dated September 30, 2019.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

X4 PHARMACEUTICALS, INC.

Date: September 30, 2019

By: /s/ Adam S. Mostafa
Adam S. Mostafa
Chief Financial Officer

X4 Pharmaceuticals Reports Positive Data from Phase 2a Trial of Mavorixafor in Combination with Axitinib in Advanced Clear Cell Renal Cell Carcinoma Patients

Subgroup exceeds target of 50% improvement in median progression-free survival

12% of patients remain on combination treatment with durations of 17 months or longer

Hosts investor conference call and webcast on Monday, September 30 at 8:00 AM EDT / 2:00 PM CEST

Barcelona — September 30, 2019 — X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, today announced positive results from the Phase 2a portion of its open-label Phase 1/2 clinical trial of mavorixafor (X4P-001) in combination with axitinib (Inlyta®) in patients with advanced clear cell renal cell carcinoma (ccRCC). Data were presented at the European Society for Medical Oncology (ESMO) 2019 Congress today in Barcelona.

Combination therapy with mavorixafor and approved tyrosine kinase inhibitor (TKI) axitinib was generally well tolerated with a manageable safety profile and demonstrated clinical improvement with encouraging median progression free survival (mPFS) in a heavily pretreated advanced ccRCC patient population. Of the 65 patients in the trial, 49 patients (or 75%) received mavorixafor + axitinib as a third- to ninth-line therapy, having received between two and eight prior therapies with a TKI, immuno-oncology (IO) agent, or other systemic therapy. Fifty-seven of the 65 patients in the trial (or 88%) had an intermediate or poor prognosis.

Overall mPFS across clinically evaluable patients receiving mavorixafor + axitinib (n=62) was 7.4 months. Predefined subpopulations examined patients with immediate prior TKI and IO treatment. Patients treated in the subgroup with immediate prior TKI therapy (n=34) demonstrated an objective response rate (ORR) of 18% and an increased mPFS of 7.4 months. This is a greater than 50% improvement from the 4.8-month historical mPFS with axitinib alone.[1] Patients treated with mavorixafor + axitinib in the subgroup with immediate prior IO therapy (n=18) had an ORR of 61% and an increased mPFS of 11.6 months. In addition, eight of the 65 patients remain on the combination therapy today, with durations of treatment of 17 months or longer. Results suggest mavorixafor may enhance clinical response to axitinib and other TKIs that target tumor angiogenesis, as well as immunotherapy agents.

“In recent years a growing number of vascular endothelial growth factor (VEGF) TKI-based therapies (e.g., axitinib + pembrolizumab), have improved outcomes for patients with ccRCC. Despite these advances, most patients eventually develop resistance to therapy, and new treatment options are necessary to meet this unmet medical need,” commented David F. McDermott, M.D., Beth Israel Deaconess Medical Center, Harvard Medical School and lead investigator of the study. “In this trial of mavorixafor, a novel CXCR4 pathway inhibitor, and axitinib in patients with metastatic ccRCC who had failed prior therapy, the combination was well tolerated and the anti-tumor activity was encouraging. We look forward to confirming the efficacy of mavorixafor in a randomized trial.”

This Phase 1/2, multi-center, open-label trial of mavorixafor in combination with axitinib included 65 patients with histologically confirmed advanced ccRCC, all of whom received at least one prior systemic therapy. The safety analyses included 65 patients from Phases 1/2 who were treated with 400 mg mavorixafor (200 mg twice daily or 400 mg once daily) + 5 mg axitinib twice daily. Treatment responses were assessed using Response Evaluation Criteria in Solid Tumor, or RECIST v1.1 (a validated set of criteria to assess changes in tumor burden), every eight weeks from day one for 80 weeks, and then every 12 weeks thereafter, by blinded, independent central review. Treatment-related serious adverse events were diarrhea, hyperkalemia and hypertension (n=2, or 3%) and blood creatinine increased, dehydration, fatigue, hepatic enzyme increase, nausea, sepsis, trachea-oesophageal fistula, and vomiting (n=1 each, or 1.5%).

“These promising results, especially among heavily pre-treated patients with poor prognoses, add to a published body of evidence supporting mavorixafor’s generally favorable safety and tolerability profile and its novel CXCR4 mechanism of action that has been shown to induce immune-mediated antitumor activity as a single agent and in combination with approved therapies,” said Lynne Kelley, M.D., FACS, Chief Medical Officer of X4 Pharmaceuticals. “We are encouraged by these data, including the eight patients who remain on combination therapy with mavorixafor and axitinib for 17 months or longer. We look forward to continuing to explore the potential benefit of mavorixafor in underserved cancer patients with solid tumors, including as a potential triple combination agent in addition to TKI and checkpoint inhibitor therapies or in combination with other standard of care treatments.”

Mavorixafor is a potentially first-in-class, once-daily, oral, small molecule antagonist of chemokine receptor CXCR4. CXCR4 signaling is thought to contribute to the lack or loss of tumor responsiveness to angiogenesis inhibitors, like axitinib. Elevated expression of CXCR4 by RCC tumors is also correlated with an overall poor prognosis. In xenograft models studied previously, mavorixafor in combination with axitinib, a VEGF receptor TKI, reduced myeloid-derived suppressor cell infiltration and proangiogenic signals, and demonstrated greater than additive antitumor activity.

Details of the investor conference call and webcast are as follows:

Time and Date: Monday, September 30 at 8:00 AM EDT / 2:00 PM CEST
US Toll-Free Dial-In Number: (866) 721-7655
International Dial-In Number: (409) 216-0009 / Spain 0934923253
Conference ID: 4787329
Webcast: A live audio webcast of the conference call may be accessed in the “Investors” section of the Company’s website at the following link: http://investors.x4pharma.com/events-and-presentations.

About Mavorixafor

X4 Pharmaceuticals' lead product candidate, mavorixafor (X4P-001), is a potentially first-in-class, once-daily, oral inhibitor of CXCR4, currently in Phase 2 development for the treatment of clear cell renal cell carcinoma (ccRCC). Mavorixafor has demonstrated single and combination agent activity and proof of mechanism in Phase 1b and Phase 2a trials, respectively, along with a favorable safety and tolerability profile. Mavorixafor is also in development for the treatment of WHIM syndrome, as well as Severe Congenital Neutropenia (SCN) and Waldenström's macroglobulinemia (WM). Mavorixafor was designated orphan drug status by the U.S. Food and Drug Administration in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome.

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and certain cancers. The company's oral small molecule drug candidates antagonize the CXCR4 pathway, which plays a central role in immune surveillance. X4's most advanced product candidate, mavorixafor (X4P-001), is in a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease, and is currently also under investigation in combination with axitinib in the Phase 2a portion of an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC). X4 is also planning to commence clinical trials of mavorixafor in Severe Congenital Neutropenia (SCN) and Waldenström's macroglobulinemia (WM) in 2019. The company was founded and is led by a team with extensive biopharmaceutical product development and commercialization expertise and is committed to advancing the development of innovative medicines on behalf of patients with limited treatment options. X4 is a global company that is headquartered in Cambridge, Massachusetts with research offices based in Vienna, Austria. For more information, please visit www.x4pharma.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements include, but are not limited to, statements regarding X4's plans for the development of mavorixafor (X4P-001) or any of X4's other product candidates or programs, including regarding the Phase 2a portion of the Phase 1/2 clinical trial of mavorixafor in combination with axitinib in ccRCC or plans to commence clinical trials of or otherwise evaluate mavorixafor as part of a triple combination therapy or in combination with any other treatments; the potential benefits of mavorixafor; the safety or efficacy of mavorixafor or any of X4's other product candidates or programs or the commercial opportunity in any target indication. These statements are subject to various risks and uncertainties, actual results could differ materially from those projected and X4 cautions investors not to place undue reliance on the forward-looking statements in this press release. These risks and uncertainties include, without limitation, the risk that trials and studies may be delayed and may not have satisfactory outcomes, potential adverse effects arising from the testing or use of mavorixafor or other product candidates, the

risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving X4's product candidates, and the risk that costs required to develop mavorixafor or other product candidates or to expand X4's operations will be higher than anticipated. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, the risks and uncertainties described in the section entitled "Risk Factors" in X4's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as updated by X4's Current Report on Form 8-K filed with the SEC on April 11, 2019, and in other filings X4 makes with the SEC from time to time. X4 undertakes no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

Investors:

Stephanie Carrington
Westwicke, an ICR company
646-277-1282
Stephanie.Carrington@icrinc.com

Media:

Darcie Robinson
Westwicke, an ICR company
203-919-7905
Darcie.robinson@icrinc.com

---

1 Rini BI et al. Comparative effectiveness of axitinib versus sorafenib in advanced renal cell carcinoma (AXIS): a randomised phase 3 trial. Lancet. 2011 Dec 3;378(9807):1931-9.

Exhibit 99.2


X4
PHARMACEUTICALS
DATA FROM PHASE 2A TRIAL OF MAVORIXAFOR IN COMBINATION WITH AXITINIB IN ADVANCED CLEAR CELL RENAL CELL CARCINOMA
ESMO 2019 CONGRESS, BARCELONA
INVESTOR CONFERENCE CALL
SEPTEMBER 30, 2019

AGENDA

- Welcome
  - Stephanie Carrington, Investor Relations
- Overview of Mavorixafor and Oncology Data
  - Lynne Kelley, MD, Chief Medical Officer
- Data from Phase 2a portion of open-label Phase 1/2 trial of mavorixafor (X4P-001) in combination with axitinib (Inlyta®) in patients with advanced clear cell renal cell carcinoma (ccRCC)
  - David F. McDermott, M.D., Beth Israel Deaconess Medical Center, Harvard Medical School and lead investigator of the trial
- Q&A Session
  - Paula Ragan, PhD, Chief Executive Officer
  - Lynne Kelley, MD, Chief Medical Officer
  - Adam Mostafa, Chief Financial Officer
  - David F. McDermott, M.D

FORWARD LOOKING STATEMENTS

This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation are forward-looking statements, including, but not limited to, statements regarding X4's plans for the development of mavorixafor (X4P-001), including plans to commence clinical trials of or otherwise evaluate mavorixafor as part of a triple combination therapy or in combination with any other treatments; the potential benefits of mavorixafor; or the safety, durability or efficacy of mavorixafor.

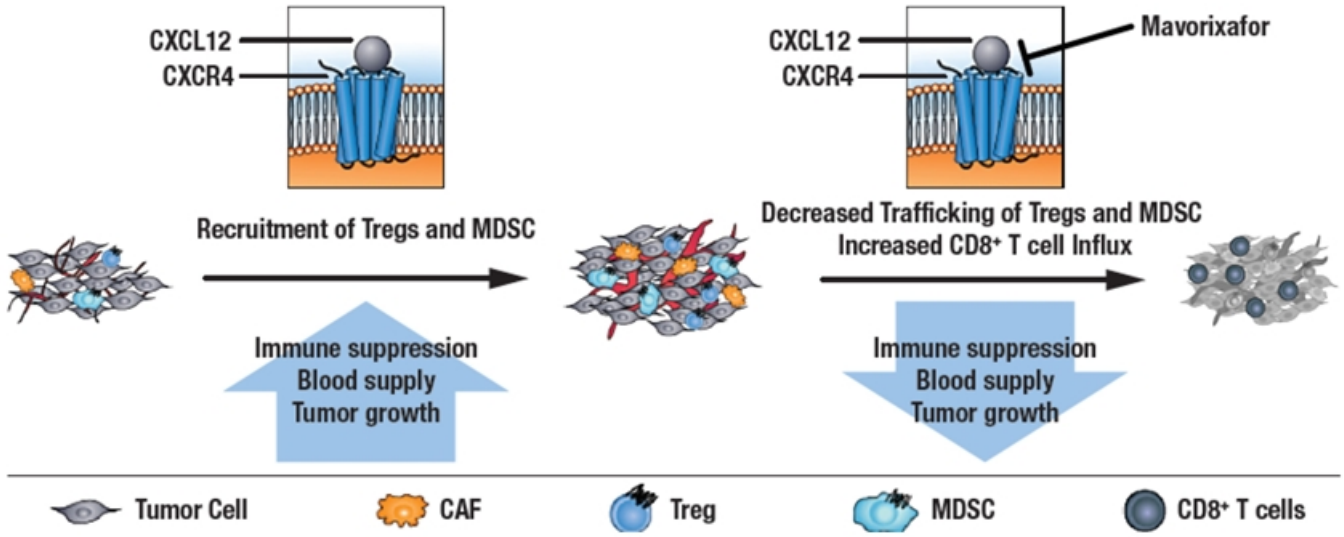
These forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. These risks and uncertainties include, without limitation, the risk that trials and studies may be delayed and may not have satisfactory outcomes, potential adverse effects arising from the testing or use of mavorixafor or other product candidates, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving X4's product candidates, the risk that costs required to develop mavorixafor or other product candidates or to expand X4's operations will be higher than anticipated. and other risks and uncertainties described in X4's filings with the Securities and Exchange Commission, including under the heading "Risk Factors" in X4's most recent Annual Report on Form 10-K filed with the SEC, as updated by X4's Current Report on Form 8-K filed with the SEC on April 11, 2019, and in subsequent filings X4 makes with the SEC. These forward-looking statements represent X4's beliefs and assumptions only as of the date of this presentation, and X4 assumes no obligation to update or revise any of these statements. X4 caution investors not to place considerable reliance on these forward-looking statements.

OVERVIEW OF MAVORIXAFOR (X4P-001)

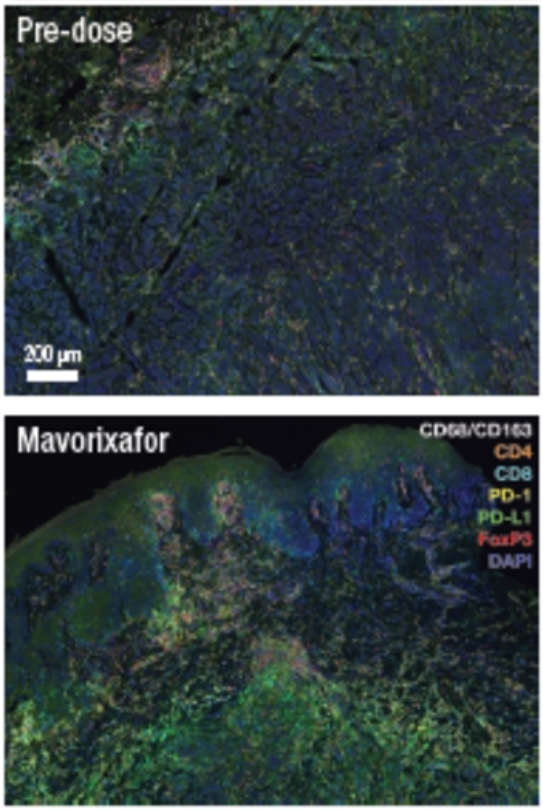
- Oral once a day small molecule CXCR4 antagonist that allosterically inhibits the binding by CXCL12/SDF1-α, the only known CXCR4 ligand[1]
- Single-agent chronic treatment with 400 mg QD of mavorixafor was observed to be well-tolerated with only Grade 1 treatment-related AEs in a Phase 2 trial of WHIM patients[2]
- Biopsies from melanoma patients treated with mavorixafor show enhanced immune cell tumor infiltration and activation leading to increases in both tumor inflammation signature scores and IFN-γ gene expression signatures[3]
- In mouse xenograft RCC models, treatment with mavorixafor in combination with axitinib demonstrates greater than additive anti-tumor activity[4]

1) Stone et al. Antimicrob Agents Chemother. 2007;51(7):2351–2358. 2) Dale et al. ASH 2018. 3) Andtbacka et al. AACR 2018. 4) Panka et al. Eur. J Cancer 2016; 69:S105.

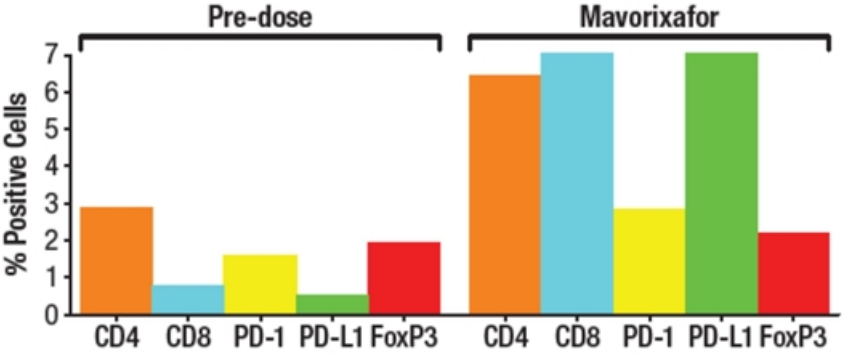
MAVORIXAFOR: MECHANISM OF ACTIONS



MAVORIXAFOR INCREASES TUMOR IMMUNE CELL INFILTRATION WITH CD8+ T-CELLS AND ACTIVATION IN PHASE 1B TRIAL



Increased CD8/FoxP3 Ratio and PD-L1 post Mavorixafor treatment

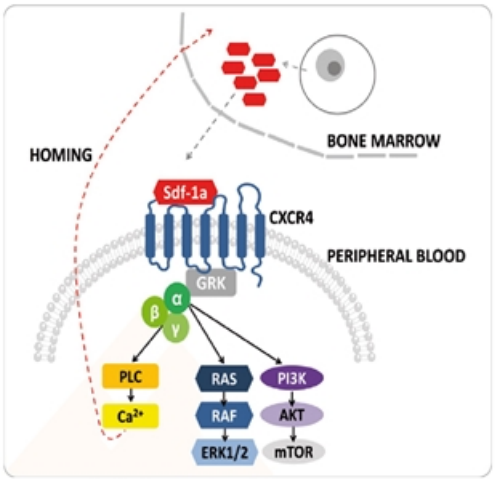


Multiplex immunohistochemistry staining of tumor samples from a melanoma patient treated with 400 mg QD mavorixafor monotherapy. Biopsy samples obtained pre-dose and after 3 weeks of mavorixafor monotherapy were immunostained with an antibody panel and analyzed using HALO™ image analysis software

(A Phase 1b Trial of X4P-001 Alone and With Pembrolizumab in Patients With Advanced Melanoma - ClinicalTrials.gov Identifier: NCT02823405)

ROLE OF CXCR4 AND RENAL CELL CARCINOMA

- Approximately 70% of sporadic clear cell renal cell carcinoma (RCC) patients have a loss of VHL gene function that drives tumor angiogenesis by increasing VEGF receptor expression[1]
- A number of tyrosine kinase inhibitors (TKIs) that target the VEGF pathway have been approved for RCC, including axitinib, although most patients will eventually relapse through angiogenic escape[2]
- CXCR4 signaling via hypoxia-induced increases in HIF1/2a is thought to contribute to the lack or loss of tumor responsiveness to angiogenesis inhibitors, like axitinib.[3]
- Elevated expression of CXCR4 by RCC tumors is also correlated with an overall poor prognosis. [4,5]



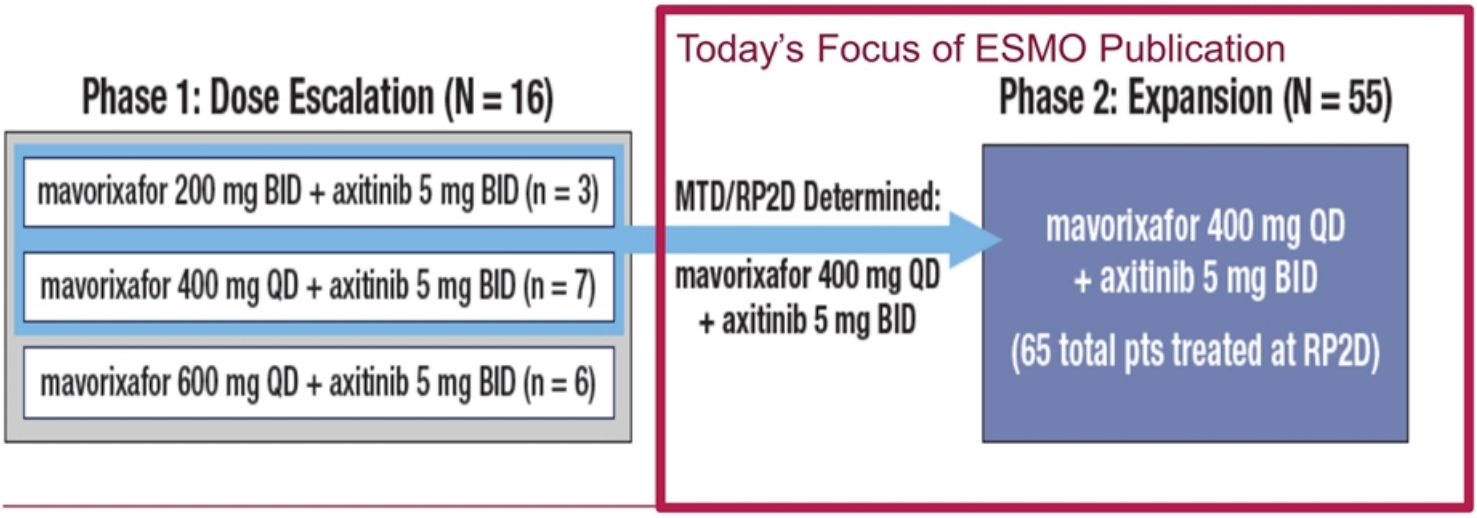
1) Maher et al. Eur J Hum Genet. 2011; 19:617-623. 2) Bellesoeur et al. Drug Des Devel Ther. 2017;11:2801-2811. 3) Vanharanta et al Nature Medicine, 2013) 4) Staller et al. Nature 2003; 425:307-311. 63:E820. 5) Sekiya et al. Human Pathology. 2012; 43:904-910

DATA FROM PHASE 2A PORTION OF OPEN-LABEL PHASE 1/2 CLINICAL TRIAL

- Poster Discussion Session at ESMO
  - Abstract #2521: Safety and Efficacy of the Oral CXCR4 Inhibitor X4P-001 + Axitinib in Advanced Renal Cell Carcinoma Patients: An Analysis of Subgroup Responses by Prior Treatment
  - Lead study investigator: David F. McDermott, M.D., Beth Israel Deaconess Medical Center, Harvard Medical School

STUDY DESIGN

- Phase 1/2, multi-center, open-label trial, n = 65 for safety analyses
- Treatment: 400 mg mavorixafor (200 mg BID or 400 mg QD) + 5 mg BID axitinib
- Patient populations: patients with histologically confirmed clear cell RCC who have received at least 1 prior systemic therapy and ECOG ≤ 2
- Treatment responses assessed using RECIST v1.1 every 8 weeks from Day 1 for 80 weeks and then every 12 weeks thereafter by blinded, independent central review



DEMOGRAPHIC AND BASELINE CHARACTERISTICS

Mavorixafor + Axitinib (N = 65)		
Demographics		
Age (Years)	Median (Range)	64
	Range	42-87
Gender	Male	55 (85%)
	Female	10 (15%)
Ethnicity	Hispanic or Latino	7 (11%)
	Not Hispanic or Latino	58 (89%)
Race	Asian	17 (26%)
	Black / African American	2 (3%)
	White	43 (66%)
	Other	3 (5%)
ECOG Status	0	25 (39%)
	1	36 (55%)
	2	4 (6%)

Baseline Characteristics		
Number of Prior Systemic Therapies	Median (Range)	2 (1-8)
	1	16 (25%)
	2	18 (28%)
	≥3	31 (48%)
Patients with:	Previous CPI therapy	31 (48%)
	Previous TKI therapy	59 (91%)
	Previous IO therapy	39 (60%)
	Any prior nephrectomy	59 (91%)
Prognosis at Baseline based on Heng score	Favorable	8 (12%)
	Intermediate	45 (69%)
	Poor	12 (19%)

Clinical cut-off date: August 27, 2019

COMBINATION TREATMENT WITH MAVORIXAFOR + AXITINIB WAS OBSERVED TO BE GENERALLY WELL-TOLERATED IN THE TRIAL WITH A MANAGEABLE SAFETY PROFILE

Adverse Events (All Grades ≥ 20% and Grade ≥ 3 in > 2 Pts) Related to Mavorixafor or Axitinib (N = 65)		
Adverse Event (Related)	**All Grades**	**Grade ≥ 3**
Diarrhea	35 (54%)	7 (11%)
Decreased Appetite	29 (45%)	6 (9%)
Fatigue	29 (45%)	3 (5%)
Hypertension	25 (39%)	14 (22%)
Nausea	19 (29%)	3 (5%)
Weight decreased	14 (22%)	2 (3%)
Dysphonia	14 (22%)	0
Blood Creatinine Increased	13 (20%)	1 (2%)
Hypothyroidism	13 (20%)	1 (2%)

Clinical cut-off date: August 27, 2019

- **Ten patients (15%) discontinued combination therapy due to treatment-related AEs (mavorixafor or axitinib) of creatinine increase (3 pts); hypertension (2 pts); and azotaemia, diarrhea, fatigue, hyperkalemia, retinal vein occlusion, sepsis, and trachea-oesophogeal fistula (1 pt each)**

COMBINATION TREATMENT WITH X4P-001 (MAVORIXAFOR) + AXITINIB DEMOSTRATED CLINICAL BENEFIT IN TRIAL IN HEAVILY PRETREATED RCC PATIENTS WITH ADVANCED DISEASE

Mavorixafor + Axitinib (N = 65)		
Baseline Characteristics		
Number of Prior Systemic Therapies	Median (Range)	2 (1-8)
	1	16 (25%)
	2	18 (28%)
	≥3	31 (48%)
Patients with:	Previous CPI therapy	31 (48%)
	Previous TKI therapy	59 (91%)
	Previous IO therapy	39 (60%)
	Any prior nephrectomy	59 (91%)
Prognosis at Baseline based on Heng score	Favorable	8 (12%)
	Intermediate	45 (69%)
	Poor	12 (19%)

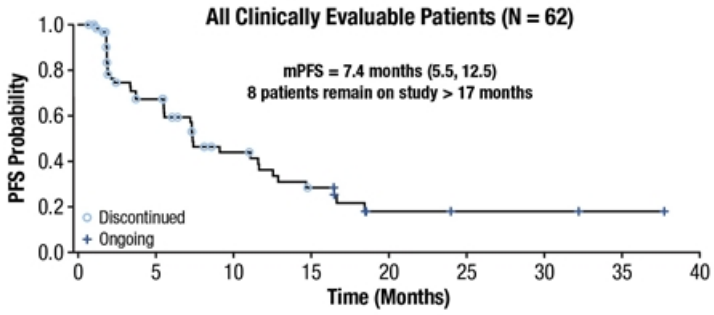
Clinical cut-off date: August 27, 2019

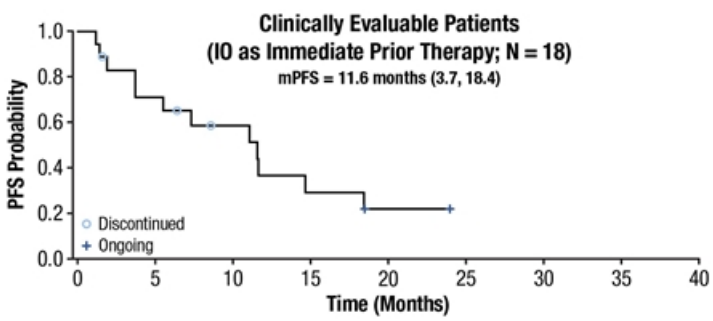
Objective Response Rates:

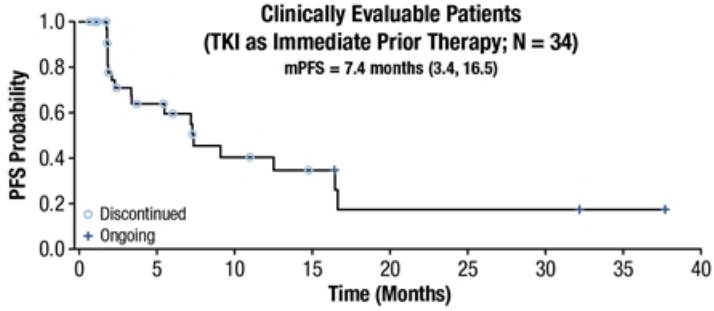
- **All clinically evaluable pts (N = 62): 29%**
- **Immediate prior TKI therapy (N = 34): 18%**
- **Immediate prior IO therapy (N = 18): 61%**

- **75% of patients has received 2 or more prior therapies**
- **88% had an intermediate or poor prognosis by Heng Score**

SUBGROUP-SPECIFIC CLINICAL BENEFIT OF MAVORIXAFOR + AXITINIB COMBINATION TREATMENT BASED ON IMMEDIATE PRIOR THERAPY RECEIVED







CONCLUSION AND FUTURE DIRECTION

Conclusion:

- Combination therapy with 400 mg QD Mavorixafor + 5 mg BID axitinib was observed in the trial to be generally well-tolerated with a manageable safety profile
- Mavorixafor + axitinib demonstrated encouraging mPFS in this heavily pretreated advanced RCC patient population
- mPFS with immediate prior IO therapy was 11.6 months
- mPFS with immediate prior TKI therapy was 7.4 months
- 8 patients remain on study > 17 months
- The results suggest that mavorixafor may enhance clinical responses to axitinib and other TKIs that target tumor angiogenesis, as well as immunotherapy agents, such as CPIs

Future Direction:

- Triple combination of mavorixafor in addition to TKI and CPI is worthy of future investigation in a larger RCC patient population, particularly in the first-line setting
- The contribution of mavorixafor to the durable responses observed should be validated in a randomized Phase II trial


X4
PHARMACEUTICALS
DATA FROM PHASE 2A TRIAL OF MAVORIXAFOR IN COMBINATION WITH AXITINIB IN ADVANCED CLEAR CELL RENAL CELL CARCINOMA
ESMO 2019 CONGRESS, BARCELONA
INVESTOR CONFERENCE CALL
SEPTEMBER 30, 2019