FEBRUARY 2021

PERSPECTIVE ON THE Rx PIPELINE

Understanding changes in the medication market and their impact on cost and care.

Elixir continuously monitors the drug pipeline. As treatment options change, we evaluate and share our perspective on the clinical benefits, cost effectiveness and overall impact to payers and members. Our Perspective on the Rx Pipeline report provides ongoing actionable insights from our team of clinical experts and the steps we are taking to protect and improve plan performance.

INCLUDED IN THIS EDITION:

- Clinical Pipeline
- Key New Drug Approvals
- New Indications
- Upcoming and Recent Generic and Biosimilar Launches
- FDA Safety Update
- Drug Shortages, Discontinuations and Recalls

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CRAFTED Rx SOLUTIONS

Clinical Pipeline

Arimoclomol

Manufacturer: Orphazyme
Indication/Use: Niemann-Pick disease
Dosage Form: Oral
Pipeline Stage: PDUFA 3/17/2021

Niemann-Pick disease type C (NPC) is a rare, progressive genetic disorder that is characterized by the body's inability to transport cholesterol and other fatty substances inside of cells. This leads to an accumulation of fatty substances, or lipids, within various tissues of the body, including the brain tissue. The onset and symptoms can vary from one individual to another, even within the same family. Childhood onset is most common, discovered as children are developing writing skills in school and having difficulty. As NPC progresses, patients may develop speech issues followed by psychologic disturbances and dementia-like symptoms. Individuals diagnosed usually do not live through their 20s or 30s. NPC occurs in approximately one in every 100,000-120,000 live births and affects both males and females equally; however, many are undiagnosed or misdiagnosed, so this number may be a misrepresentation in the population. NPC is part of a larger group of other disorders that affect lysosomal storage in the cells. Current treatment is directed towards symptom management.[1]

Arimoclomol would be the first FDA-approved treatment for NPC. A phase II/III prospective, randomized, double blind, placebo-controlled trial evaluated the efficacy and safety of arimoclomol with a small study size of 50 with this orphan disease. Trial data showed arimoclomol was well tolerated and there was evidence of slowed disease progression during the two years of the study.[2-3]

Arimoclomol also has additional indications in the drug pipeline for amyotrophic lateral sclerosis (ALS), Gaucher disease and inclusion body myositis. Data has not been released for these disease states yet. That data is expected to be released in the first half of 2021. If this product is approved by the FDA for NPC, it will most likely require utilization management to confirm that appropriate data is available to support use of arimoclomol.[2]

Glossary of Terms **BLA** - Biologics License Application **NDA** - New Drug Application **PDUFA** - Prescription Drug User Fee Act

Clinical Pipeline

KD025 *Belumosudil*

Manufacturer: Kadmon
Indication/Use: Graft versus host disease
Dosage Form: Oral
Pipeline Stage: PDUFA 5/30/2021

Graft versus host disease (GVHD) is a common complication of allogenic hematopoietic cell transplant (HCT) that can occur regardless of immunosuppressive agents. The disease is a result of a non-identical, donor-immune cell recognizing the host as foreign and causing an immune reaction.[4] GVHD can be chronic or acute. Chronic GVHD, or cGVHD, can present at any time post HCT and can impact a wide range of organs, primarily the skin, liver, gastrointestinal tract and lungs. Risk factors for cGVHD include prior acute GVHD, older patient age, mismatched and unrelated donors, female donor for male recipient, and grafting with growth factor-mobilized blood cells.[5]

Initial GVHD therapy may be based on organs involved, prophylaxis and symptom severity. More severe cases may require systemic glucocorticoids. Despite corticosteroid treatment, approximately 40 to 50% of GVHD patients develop steroid-refractory disease.[6] Treatment-resistant disease may require a calcineurin inhibitor (cyclosporine or tacrolimus). Other FDA-approved drugs for GVHD include ruxolitinib (acute GVHD), ibrutinib and rituximab. Tyrosine kinase inhibitors, such as imatinib are also used. Patients with extensive GVHD have poor long-term outcomes and most of the morbidity and mortality associated with cGVHD is a complication of immune suppression.

Kadmon will shortly be seeking FDA approval for belumosudil as a treatment for cGVHD, as well as several other indications including pulmonary fibrosis, psoriasis vulgaris and systemic sclerosis.[4, 7] Belumosudil is a selective inhibitor of Rho-associated coiled-coil kinase 2 (ROCK2). ROCK inhibitors are approved as ophthalmic treatments for glaucoma, but no oral options currently are FDA approved. The ROCK pathway modulates inflammatory responses and may also have a role in the pathogenesis of many fibrotic or immune diseases.[4, 7] Belumosudil has received FDA Breakthrough Therapy Designation and Orphan Drug Designation for GVHD.

Belumosudil has a phase II open-label study (Rockstar study KD025-213) consisting of 132 patients 12 years of age and older with cGVHD who received two to five prior lines of systemic therapy. These patients received glucocorticoid therapy with a stable dose over the two weeks prior to screening. Current treatment with ibrutinib was not allowed, but patients could be on concomitant corticosteroids, calcineurin inhibitors rituximab, etc. Patients were randomized 1:1 to receive belumosudil 200 mg once daily or twice daily and evaluated for an overall response rate (ORR) as defined by the 2014 NIH Consensus Development Project on clinical trials in cGVHD. ORR were 73% (95% CI, 60%-83%, $p<0.0001$) for once daily treatment versus 74% (95% CI, 62%-84%, $p<0.0001$) for twice daily treatment. Estimated primary completion date of the Rockstar study is September of 2021.[8, 9] Kadmon has stated previously that belumosudil has been well tolerated by patients in current studies.

Belumosudil is not intended to be first-line therapy, but may join Imbruvica® as another FDA-approved treatment for cGVHD, a notoriously difficult disease to treat. Prior authorization would be appropriate for such a specific indication in a rare disease. The unique mechanism of action and subsequent indications that Kadmon is seeking for belumosudil may result in more substantial market share, if approvals are granted.

Key New Drug Approvals

Gemtesa® *vibegron*

Manufacturer: Urovant Sciences, Inc.
Indication/Use: Overactive bladder (OAB)
Dosage Form: Oral tablets
Traditional or Specialty: Traditional

Gemtesa was approved by the FDA on December 23, 2020, for treatment of OAB with symptoms of urge urinary incontinence, urgency and urinary frequency in adults. It is the second beta 3 agonist to come to market and first OAB product approved since 2012. This product will compete with Myrbetriq® (mirabegron) for use in overactive bladder.

For more information: https://gemtesa.com/

Imcivree™ *setmelanotide*

Manufacturer: Rhythm Pharmaceuticals, Inc.
Indication/Use: Chronic weight management
Dosage Form: Subcutaneous injection
Traditional or Specialty: Specialty

Imcivree was granted FDA approval on November 25, 2020, for weight management in individuals six years of age and older with obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency confirmed by genetic testing, demonstrating variants in POMC, PCSK1 or LEPR genes that are interpreted as pathogenic, likely pathogenic or of uncertain significance (VUS). Current pharmacological weight loss treatments are limited for genetic obesity disorders and Imcivree may offer an option other than bariatric surgery. Imcivree does have a lengthy side effect profile with multiple warnings, including disturbance in sexual arousal, depression and suicidal ideation, skin pigmentation, and darkening of pre-existing nevi.

For more information: https://blog.elixirsolutions.com/perspective-on-the-rx-pipeline-november-2020

Klisyri® *tirbanibulin*

Manufacturer: Almirall, LLC
Indication/Use: Actinic keratosis on the face or scalp
Dosage Form: Topical ointment
Traditional or Specialty: Traditional

Klisyri topical ointment offers a new mechanism of action in the pharmacological treatment of actinic keratosis (AK). The microtubule inhibitor has a short duration of treatment and possibly milder side effect profile than some current therapy. FDA approval was granted on December 14, 2020, for the self-administered novel AK treatment.

For more information: https://www.almirall.com/newsroom/news/almirall-announces-fda-approval-of-klisyri%C2%AE-tirbanibulin-a-new-innovative-topical-treatment-for-actinic-keratosis

Key New Drug Approvals

Orgovyx™ *relugolix*

Manufacturer: Myovant Sciences, Inc.
Indication/Use: Advanced prostate cancer
Dosage Form: Oral tablets
Traditional or Specialty: Specialty

The FDA approved Orgovyx on December 18, 2020, to treat the second most common cancer diagnosis in men, prostate cancer. Orgovyx will compete with other androgen deprivation therapies (ADT), such as Lupron Depot®, but offers an oral treatment option. Orgovyx is a gonadotropin releasing hormone (GnRH) antagonist, which differs from leuprolide, a GnRH agonist. Continued safety data on cardiovascular risk with Orgovyx compared to other ADT may help define its place in therapy.

For more information: https://www.fda.gov/news-events/press-announcements/fda-approves-first-oral-hormone-therapy-treating-advanced-prostate-cancer

Orladeyo™ *berotralstat*

Manufacturer: BioCryst Pharmaceuticals, Inc.
Indication/Use: Prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 12 years of age and older
Dosage Form: Oral capsule
Traditional or Specialty: Specialty

Orladeyo was approved on December 4, 2020, as the first oral preventive therapy for HAE attacks. Orladeyo is a kallikrein inhibitor, much like subcutaneous Takhzyro®.

For more information: https://blog.elixirsolutions.com/perspective-on-the-rx-pipeline-november-2020

Key New Drug Approvals

Oxlumo™ *lumasiran*

Manufacturer: Alnylam Pharmaceuticals, Inc.
Indication/Use: Primary hyperoxaluria type 1 (PH1)
Dosage Form: Subcutaneous injection
Traditional or Specialty: Specialty

On November 23, 2020, Oxlumo was granted FDA approval for the treatment of PH1, an ultra-rare genetic disease that leads to an overproduction in oxalate. Oxlumo is intended to lower urinary oxalate levels in pediatric and adult patients. It is estimated by the National Organization for Rare Disorders (NORD) that there are one to three cases per 1 million people in the U.S. with PH1.[10] Patients often present with kidney and urinary stones and, if left untreated, the disease can ultimately lead to advanced renal disease or cause deposits in various organs, such as the heart. Oxlumo is intended to be given in a physician office or infusion center.

For more information: https://www.fda.gov/news-events/press-announcements/fda-approves-first-drug-treat-rare-metabolic-disorder

Zokinvy™ *ionafarnib*

Manufacturer: Eiger Biopharmaceuticals
Indication/Use: Reduction of risk of mortality in Hutchinson-Gilford Progeria Syndrome and for treatment of processing-deficient Progeroid Laminopathies
Dosage Form: Oral capsule
Traditional or Specialty: Specialty

Zokinvy was FDA approved on November 20, 2020 for treatment of progeria in patients 12 months of age and older with a body surface area of 0.39m^2. Candidates for therapy should either have the heterozygous LMNA mutation with progerin-like protein accumulation or homozygous or compound heterozygous ZMPSTE24 mutations. This is the first drug approved for progeria, a disease that causes premature aging and patients to pass away in their teenage years.

For more information: https://www.zokinvy.com/

Key New Drug Approvals

VERQUVO® *vericiguat*

Manufacturer: Merck & Co; Bayer
Indication/Use: Reduce the risk of cardiovascular death and heart failure hospitalization following a hospitalization for heart failure or need for outpatient IV diuretics
Dosage Form: Oral
Traditional or Specialty: Traditional

VERQUVO received FDA approval for the treatment of heart failure on January 19, 2021.

In 2018, approximately 380,000 (13.4%) death certificates mentioned heart failure (HF). Heart failure management and hospitalizations place a large burden on the healthcare system, with 6.2 million adults living with the condition. Rates of HF re-hospitalization or cardiovascular (CV) death are greatest in those previously hospitalized with HF.[11] VERQUVO was granted priority review by the FDA and since approved to reduce the risk of CV death and hospitalization following a worsening HF event in patients with symptomatic chronic HF with reduced ejection fraction (HFrEF).[12] This medication will be given in conjunction with other medications to control heart failure symptoms.

The VICTORIA (Vericiguat Global Study in Subjects with Heart Failure with Reduced Ejection Fraction) trial evaluated this novel mechanism of action as an add-on therapy to standard of care. This trial was designed to include sicker HF patients that had been hospitalized in the last six months or treated with an intravenous diuretic. Most of the study participants had been hospitalized within the last three months of enrollment. The primary endpoint was a composite of death from CV causes or first hospitalization for HF compared to those taking placebo. Over the 10.8 months of the trial, 35.5% of participants in the VERQUVO group experienced a primary outcome event compared with 38.5% in the placebo group. This is statistically a hazard ratio of 0.9 (CI 0.82-0.98; p=0.02), meaning patients treated with VERQUVO were 10% less likely to experience the primary outcome. VERQUVO has a similar adverse event profile to other add-on therapies for HF management, including hypotension.[13]

VERQUVO has a unique mechanism of action, but will compete with other add-on therapies for HF, including Entresto® (sacubitril/valsartan) and Farxiga® (dapagliflozin), which are both indicated for reducing risk of CV death and hospitalization in adults with HFrEF.[14, 15] Jardiance® (empagliflozin) has an approved indication for reduced death in adults with HFrEF.[16] It is anticipated that VERQUVO will compete with at least Entresto for market share. Pricing will not be available until after market launch, but it is anticipated that VERQUVO will be priced competitively with Entresto. VERQUVO may have an advantage with less lab monitoring; however, providers will have more practice experience with Entresto. Healthcare dollars for heart failure management are significant and an additional drug mechanism in the toolbox will allow providers to help reduce hospital administration and the continued taxing of the healthcare system.

COVID-19 Vaccinations with Emergency Use Authorzation (EUA)

Manufacturer	EUA Granted	Dosing Regimen	Storage	Vaccine Efficacy	Authorized Use
Moderna[20]	12/18/20	2 doses, 28 days apart	Short term: 2° to 8°C for up to 30 days Long term: 20°C for up to 6 months	94.1%	For the prevention of coronavirus disease 2019 (COVID-19) in individuals 18 years of age and older
Pfizer/BioNTech[21]	12/11/20	2 doses, 21 days apart	-70 °C for up to 6 months Dry ice storage to maintain temperature	95%	For the prevention of coronavirus disease 2019 (COVID-19) in individuals 16 years of age and older

As of January 22, 2021, Elixir is awaiting the phase III data on additional vaccines manufactured by AstraZenaca and Janssen/Johnson & Johnson. These candidates are adenovirus vaccines and may offer an easier storage than currently EUA-approved mRNA vaccines. The EUA review timeline is expected in spring 2021.

New Indications

Benlysta® *belimumab*

Manufacturer: GlaxoSmithKline
Indication/Use: Active, autoantibody-positive systemic lupus erythematosus in those 5 years of age and older
Dosage Form: Intravenous infusion
Traditional or Specialty: Specialty
Date of Original Approval: March 10, 2011

Lupus nephritis is a kidney disease caused by lupus, which may result in dialysis or kidney transplant if the kidneys fail.[17] On December 16, 2020, the FDA expanded Benlysta's indication to adult patients with active lupus nephritis who are receiving standard therapy. Lupus nephritis is very common in children with lupus, affecting eight out of 10, and affects five out of 10 adults with the condition.[18]

For more information: https://www.benlysta.com/about-benlysta-for-lupus/lupus-nephritis/

Gavreto™ *pralsetinib*

Manufacturer: Blueprint Medicines Corporation
Indication/Use: Metastatic rearranged during transfection (RET) fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA-approved test
Dosage Form: Oral capsules
Traditional or Specialty: Specialty
Date of Original Approval: September 4, 2020

On December 1, 2020, Gavreto's label was expanded to include patients 12 years of age and older with advanced medullary thyroid cancer (MTC) or MTC that has spread who require systemic therapy AND advanced thyroid cancer or thyroid cancer that has spread who require systemic therapy and who are radioactive iodine-refractory. Gavreto's indications now include MTC and NSCLC, much like Retevmo, another RET inhibitor approved in 2020.

For more information: https://www.gavreto.com/

New Indications

Hetlioz® *tasimelteon*

Manufacturer: Vanda Pharmaceuticals, Inc.
Indication/Use: Non-24-Hour Sleep-Wake Disorder (Non-24)
Dosage Form: Oral capsules and suspension
Traditional or Specialty: Specialty
Date of Original Approval: April 4, 2014

Hetlioz received an additional indication for treatment of nighttime sleep disturbances in Smith-Magenis Syndrome (SMS) patients 16 years of age and older on December 1, 2020. Liquid formulation (Hetlioz LQ) is indicated for individuals 3 to 15 years of age. Patients with SMS may be tired during the day and have trouble falling asleep at night, with multiple awake periods beginning at an early age.

For more information: https://hetlioz.com/

Wakix® *pitolisant*

Manufacturer: Harmony Biosciences
Indication/Use: Excessive daytime sleepiness (EDS) in adult patients with narcolepsy
Dosage Form: Oral tablets
Traditional or Specialty: Traditional
Date of Original Approval: August 14, 2019

Wakix was originally FDA approved for narcolepsy in 2019. Narcolepsy may be type 1, which consists of episodes of cataplexy, or type 2, which does not. Wakix gained a cataplexy indication for adult patients with narcolepsy on October 13, 2020. Other treatments for cataplexy include sodium oxybate and antidepressants.[19]

For more information: https://aasm.org/fda-approves-wakix-for-the-treatment-of-narcolepsy/

New Indications

Xofluza® *baloxavir marboxil*

Manufacturer: Genetech, Inc.
Indication/Use: Acute uncomplicated influenza in patients 12 years of age and older who have been symptomatic for no more than 48 hours and who are otherwise healthy or at high risk of developing influenza-related complications
Dosage Form: Oral tablet/suspension
Traditional or Specialty: Traditional
Date of Original Approval: October 24, 2018

On November 24, 2020, Xofluza received the updated indication of post-exposure prophylaxis of influenza in patients 12 years and older following contact with an individual who has influenza. Tamiflu® (oseltamivir) may also be used for post exposure prophylaxis, but the specific FDA-approved indication and age range vary slightly. Notably, Xofluza is now also approved as an oral suspension.

For more information: https://www.xofluza.com/about-xofluza.html

Xolair® *omalizumab*

Manufacturer: Genentech, Inc.
Indication/Use: Moderate to severe persistent asthma in patients 6 years of age and older with a positive skin test or in vitro reactivity to a perennial aeroallergen and symptoms that are inadequately controlled with inhaled corticosteroids and chronic idiopathic urticaria in adults and adolescents 12 years of age and older who remain symptomatic despite H1 antihistamine treatment
Dosage Form: Subcutaneous injection
Traditional or Specialty: Specialty
Date of Original Approval: September 28, 2018

On November 30, 2020, the FDA granted the indication of add-on maintenance treatment of nasal polyps in adult patients 18 years of age and older with inadequate response to nasal corticosteroids. Xolair blocks IgE, which may contribute to inflammation that impacts nasal polyps.

For more information: https://www.xolair.com/nasal-polyps.html

Upcoming and Recent Generic and Biosimilar Launches

Brand Name	Generic Name	# of Manufacturer Entrants	Indication	Launched or Potential Launch Date
Amitiza®	lubiprostone	1	Chronic idiopathic constipation, irritable bowel syndrome with constipation, opioid-induced constipation	Launched
Vivlodex®	meloxicam	1	Osteoarthritis pain	Launched
Zytiga® (500 mg)	abiraterone acetate	1	Prostate cancer, metastatic	Launched
Saphris®	asenapine maleate	4	Bipolar disorder, schizophrenia	Launched
Ofirmev®	acetaminophen	3	Mild to severe pain, fever reduction	Launched
Entereg®	alvimopan	1	Postoperative ileus	Launched
Alinia® (tablet)	nitazoxanide	1	Diarrhea, infectious	Launched
Nyvepria™ (Biosimilar for Neulasta)	pegfilgrastim	1	Hematopoietic radiation injury syndrome, acute; prevention of chemotherapy-induced neutropenia	Launched
Crixivan®	indinavir sulfate	TBD	HIV-1 infection	02/10/2021
Northera®	droxidopa	5+	Neurogenic orthostatic hypotension	02/18/2021
Enjuvia®	estrogens, conjugated synthetic B	TBD	Treatment of moderate to severe vasomotor symptoms associated with menopause, moderate to severe vaginal dryness and pain with intercourse, symptoms of vulvar and vaginal atrophy associated with menopause	Launched

Upcoming and Recent Generic and Biosimilar Launches

Brand Name	Generic Name	# of Manufacturer Entrants	Indication	Potential Launch Date
Impavido®	miltefosine	TBD	Leishmaniasis	03/19/2021
Absorica®	isotretinoin	TBD	Acne, severe recalcitrant nodular	1Q 2021
Glucagon (Lilly)	glucagon	TBD	Hypoglycemia	1Q 2021
Pomalyst®	pomalidomide	5+	Kaposi sarcoma; multiple myeloma, relapsed/refractory	2Q 2021
Truvada® (100 mg/150 mg, 133 mg/200 mg, 167 mg/250 mg)	emtricitabine; tenofovir disoproxil fumarate	1	HIV-1 infection	1H 2021
Chantix®	varenicline tartrate	4	Smoking cessation	1H 2021
Velphoro®	sucroferric oxyhydroxide	TBD	Hyperphosphatemia	1H 2021

FDA Safety Updates

Drug Safety Communication

Risk of False Results with the Curative SARS-Cov-2 Test for COVID-19

On January 4, 2021, the FDA announced that Curative SARS-Cov-2 test may result in a false negative. The prescription-only Curative SARS-Cov-2 assay is a real-time RT-PCR test for SARS-Cov-2. The RT-PCR test is limited to symptomatic individuals within 14 days of COVID-19 onset, must be administered by a trained healthcare worker at a collection site, and, if a negative test resulted, it should not be the sole deciding factor for patient management. The FDA reiterated that healthcare workers should refer to the EUA summary for medical devices for information on proper use.

For more information:
https://www.fda.gov/medical-devices/safety-communications/risk-false-results-curative-sars-cov-2-test-covid-19-fda-safety-communication

https://www.cdc.gov/csels/dls/locs/2021/fda_issues_safety_communication_about_risk_of_false_results_with_curative_sars-cov-2_test_for_covid-19.html

Drug Shortages, Discontinuations and Recalls

The Coronavirus Aid, Relief and Economic Security Act (CARES Act) and FDA Shortages

Part of the CARES Act was intended to improve the FDA's capabilities of identifying, preventing and mitigating future drug shortages. The following three key modifications were presented by the FDA to accomplish this:

1. Manufacturers of certain drugs must notify the FDA if they intend to permanently discontinue production or have interruptions in manufacturing that could lead to supply disruption
2. Manufacturers of drugs, active pharmaceutical ingredients or medical devices are required to develop, implement and maintain a risk management plan
3. Those registered under section 510 of the FD&C Act must annually report the amount of each drug created for commercial distribution

These modifications should provide the FDA with more visibility into drug supply chains to help limit shortages in the future.

For more information:
https://www.fda.gov/drugs/drug-shortages/cders-coronavirus-aid-relief-and-economic-security-act-cares-act-drug-shortage-mitigation-efforts

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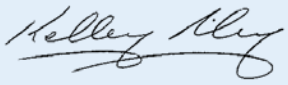
Clinical efficacy and safety, balanced with a drug's value, are always at the forefront in the Elixir formulary decisions and pipeline planning. The rationale for those decisions may go beyond the use of the FDA's labeled indication. Our clinical reviews may utilize, but are not limited to, recognized consensus guidelines, the Institute for Clinical and Economic Review (ICER), and compendium such as the National Comprehensive Cancer Network (NCCN Guidelines®) and DRUGDEX®. Elixir monitors FDA updates and safety announcements daily, as well as follows guidance from the Center of Disease Control and Prevention (CDC) and the U.S. Preventive Service Task Force (USPSTF®).

Our Clinical Steering Committee

The Elixir Clinical Steering Committee brings together leaders from across our national pharmacy care company to monitor the drug landscape, provide recommendations on how to address changes, and to ensure our clients and patients are prepared—in advance.

With any new development, we partner with our Pharmacy & Therapeutics (P&T) committee and consult with our best-in-class specialty pharmacy, to provide a balanced perspective on the clinical effectiveness of all available options, the cost impact to our plan sponsors and patients, and the impact on the overall patient experience.



Kel Riley, MD
Chief Medical Officer

More ways to improve member and plan outcomes

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