New Drugs :

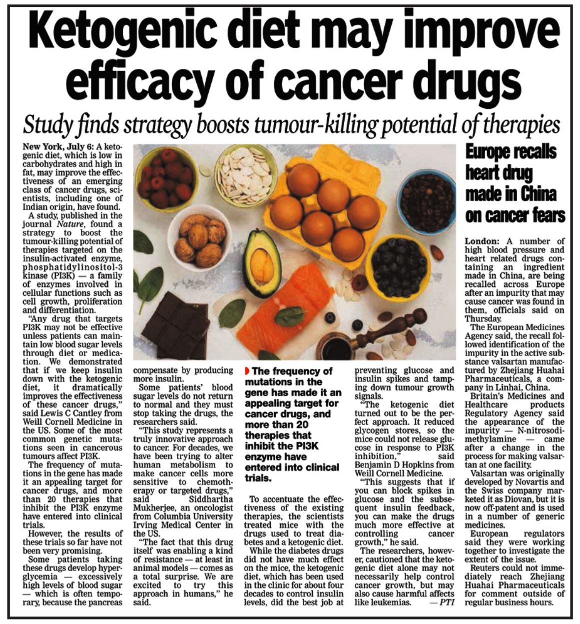
SYMDEKO : (tezacaftor; ivacaftor)

Dose : 150MG,N/A;150MG, 100MG.

Dosage form : TABLET, TABLET;ORALOne tablet, a combination of two drugs (tezacaftor and ivacaftor) is taken by mouth in the morning. The second tablet (ivacaftor) is taken by mouth, in the evening.

Use : CF is a serious genetic disorder that results in the formation of thick mucus that builds up in the lungs and other parts of the body. This can lead to severe breathing problems SYMDEKO improved lung function by allowing air to move easier. The amount of air that can be forcibly blown out in one second [percent predicted forced expiratory volume (ppFEV1)] was increased and also used to treat cystic fibrosis in patients age 12 years and older, who have specific gene mutations.

Adverse effects : Serious side effects including increased liver enzymes and clouding of the lens in the eye (cataracts).
The most commonly reported side effects associated with SYMDEKO are headache, nasopharyngitis, nausea, sinus congestion, and dizziness.



Ketogenic diet may improve efficacy of cancer drugs

Study finds strategy boosts tumour-killing potential of therapies

New York, July 6: A ketogenic diet, which is low in carbohydrates and high in fat, may improve the effectiveness of an emerging class of cancer drugs, scientists, including one of Indian origin, have found.

A study, published in the journal *Nature*, found a strategy to boost the tumour-killing potential of therapies targeted on the insulin-activated enzyme, phosphatidylinositol-3 kinase (PI3K) — a family of enzymes involved in cellular functions such as cell growth, proliferation and differentiation.

"Any drug that targets PI3K may not be effective unless patients can maintain low blood sugar levels through diet or medication. We demonstrated that if we keep insulin down with the ketogenic diet, it dramatically improves the effectiveness of these cancer drugs," said Lewis C Cantley from Weill Cornell Medicine in the US. Some of the most common genetic mutations seen in cancerous tumours affect PI3K.

The frequency of mutations in the gene has made it an appealing target for cancer drugs, and more than 20 therapies that inhibit the PI3K enzyme have entered into clinical trials.

However, the results of these trials so far have not been very promising.

Some patients taking these drugs develop hyperglycemia — excessively high levels of blood sugar — which is often temporary, because the pancreas compensate by producing more insulin.

Some patients' blood sugar levels do not return to normal and they must stop taking the drugs, the researchers said.

"This study represents a truly innovative approach to cancer. For decades, we have been trying to alter human metabolism to make cancer cells more sensitive to chemotherapy or targeted drugs," said Siddhartha Mukherjee, an oncologist from Columbia University Irving Medical Center in the US.

"The fact that this drug itself was enabling a kind of resistance — at least in animal models — comes as a total surprise. We are excited to try this approach in humans," he said.

▸ The frequency of mutations in the gene has made it an appealing target for cancer drugs, and more than 20 therapies that inhibit the PI3K enzyme have entered into clinical trials.

To accentuate the effectiveness of the existing therapies, the scientists treated mice with the drugs used to treat diabetes and a ketogenic diet.

While the diabetes drugs did not have much effect on the mice, the ketogenic diet, which has been used in the clinic for about four decades to control insulin levels, did the best job at preventing glucose and insulin spikes and tamping down tumour growth signals.

"The ketogenic diet turned out to be the perfect approach. It reduced glycogen stores, so the mice could not release glucose in response to PI3K inhibition," said Benjamin D Hopkins from Weill Cornell Medicine.

"This suggests that if you can block spikes in glucose and the subsequent insulin feedback, you can make the drugs much more effective at controlling cancer growth," he said.

The researchers, however, cautioned that the ketogenic diet alone may not necessarily help control cancer growth, but may also cause harmful affects like leukemias. — *PTI*

Europe recalls heart drug made in China on cancer fears

London: A number of high blood pressure and heart related drugs containing an ingredient made in China, are being recalled across Europe after an impurity that may cause cancer was found in them, officials said on Thursday.

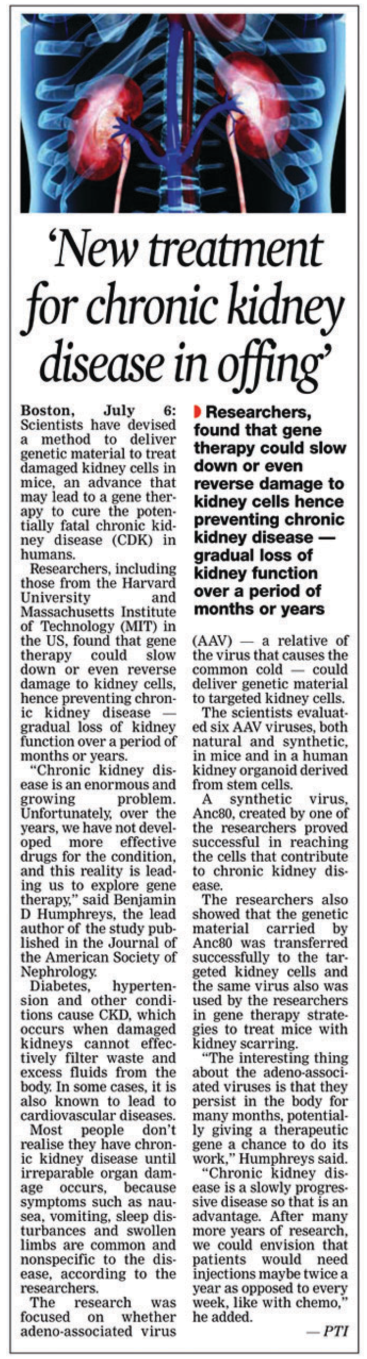
The European Medicines Agency said, the recall followed identification of the impurity in the active substance valsartan manufactured by Zhejiang Huahai Pharmaceuticals, a company in Linhai, China.

Britain's Medicines and Healthcare products Regulatory Agency said the appearance of the impurity — N-nitrosodimethylamine — came after a change in the process for making valsartan at one facility.

Valsartan was originally developed by Novartis and the Swiss company marketed it as Diovan, but it is now off-patent and is used in a number of generic medicines.

European regulators said they were working together to investigate the extent of the issue.

Reuters could not immediately reach Zhejiang Huahai Pharmaceuticals for comment outside of regular business hours.



'New treatment for chronic kidney disease in offing'

Boston, July 6: Scientists have devised a method to deliver genetic material to treat damaged kidney cells in mice, an advance that may lead to a gene therapy to cure the potentially fatal chronic kidney disease (CDK) in humans.

Researchers, including those from the Harvard University and Massachusetts Institute of Technology (MIT) in the US, found that gene therapy could slow down or even reverse damage to kidney cells, hence preventing chronic kidney disease — gradual loss of kidney function over a period of months or years.

"Chronic kidney disease is an enormous and growing problem. Unfortunately, over the years, we have not developed more effective drugs for the condition, and this reality is leading us to explore gene therapy," said Benjamin D Humphreys, the lead author of the study published in the Journal of the American Society of Nephrology.

Diabetes, hypertension and other conditions cause CKD, which occurs when damaged kidneys cannot effectively filter waste and excess fluids from the body. In some cases, it is also known to lead to cardiovascular diseases.

Most people don't realise they have chronic kidney disease until irreparable organ damage occurs, because symptoms such as nausea, vomiting, sleep disturbances and swollen limbs are common and nonspecific to the disease, according to the researchers.

The research was focused on whether adeno-associated virus (AAV) — a relative of the virus that causes the common cold — could deliver genetic material to targeted kidney cells.

▸ Researchers, found that gene therapy could slow down or even reverse damage to kidney cells hence preventing chronic kidney disease — gradual loss of kidney function over a period of months or years

The scientists evaluated six AAV viruses, both natural and synthetic, in mice and in a human kidney organoid derived from stem cells.

A synthetic virus, Anc80, created by one of the researchers proved successful in reaching the cells that contribute to chronic kidney disease.

The researchers also showed that the genetic material carried by Anc80 was transferred successfully to the targeted kidney cells and the same virus also was used by the researchers in gene therapy strategies to treat mice with kidney scarring.

"The interesting thing about the adeno-associated viruses is that they persist in the body for many months, potentially giving a therapeutic gene a chance to do its work," Humphreys said.

"Chronic kidney disease is a slowly progressive disease so that is an advantage. After many more years of research, we could envision that patients would need injections maybe twice a year as opposed to every week, like with chemo," he added. — *PTI*

ERLEADA : (Apalutamide)

Dose : 60MG

Dosage form : TABLET; ORAL Four tablets (total of 240 mg) are taken once daily with or without food.

Use : To treat a certain type of prostate cancer using novel clinical trial endpoint. ERLEADA is a drug for the treatment of prostate cancer that has not spread to other parts of the body (non-metastatic) and no longer responds to a medical or surgical treatment that lowers testosterone (castration-resistant). In the trial, ERLEADA increased the patients' survival during which there was no spreading of the cancer (called metastasis-free survival).

Adverse effect : Serious side effects including falls and bone fractures, and seizures.The most common side effects of ERLEADA includes feeling tired, elevated blood pressure, rash, and diarrhea.

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FIBRODYSPLASIA OSSIFICANS PROGRESSIVA

Introduction:

Fibrodysplasia ossificans progressiva (FOP) is a disorder in which muscle tissue and connective tissue such as tendons and ligaments are gradually replaced by bone (ossified), forming bone outside the skeleton (extra-skeletal or heterotopic bone) that constrains movement. This process generally becomes noticeable in early childhood, starting with the neck and shoulders and proceeding down the body and into the limbs.

Etiology :

Fibrodysplasia ossificans progressiva (FOP) is an extremely rare connective tissue disease. The disease is caused by a mutation of the body's repair mechanism, which causes fibrous tissue (including muscle, tendon, and ligament) to be ossified spontaneously or when damaged. In many cases, injuries can cause joints to become permanently frozen in place. Surgical removal of the extra bone growths has been shown to cause the body to "repair" the affected area with even more bone.

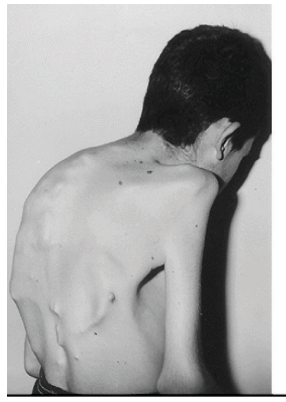
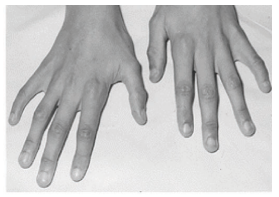
Signs and symptoms:

Fibrous dysplasia may cause few or no signs and symptoms, particularly if the condition is mild.

More severe fibrous dysplasia may cause:

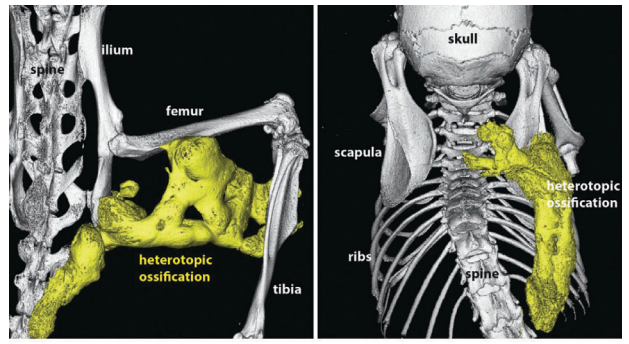
- Bone pain
- Bone deformities
- Fractures
- Nerve entrapment




Rarely, fibrous dysplasia may be associated with abnormalities in the hormone-producing glands of your endocrine system. These abnormalities may include:

- Very early puberty
- Thyroid gland problems
- Light brown spots on the skin



Consult a doctor if the child is having severe bone pain that:

- Increases with weight-bearing activity
- Interrupts sleep
- Doesn't go away with rest and causes a lump.

Since the disease is so rare, the symptoms are often misdiagnosed as cancer or fibrosis. This leads physicians to order biopsies, which can exacerbate the growth of these lumps. However, those born with FOP tend to have malformed toes or thumbs which help distinguish this disorder from other skeletal problems.

Pathophysiology :

Extra-skeletal bone formation causes progressive loss of mobility as the joints become affected. Inability to fully open the mouth may cause difficulty in speaking and eating. Over time, people with this disorder may experience malnutrition due to their eating problems. They may also have breathing difficulties as a result of extra bone formation around the rib cage that restricts expansion of the lungs.

Any trauma to the muscles of an individual with fibrodysplasiaossificansprogressiva, such as a fall or invasive medical procedures, may trigger episodes of muscle swelling and inflammation (myositis) followed by more rapid ossification in the injured area. Flare-ups may also be caused by viral illnesses such as influenza.

People with fibrodysplasiaossificansprogressiva are generally born with malformed big toes. This abnormality of the big toes is a characteristic feature that helps to distinguish this disorder from other bone and muscle problems. Affected individuals may also have short thumbs and other skeletal abnormalities.

Complications:

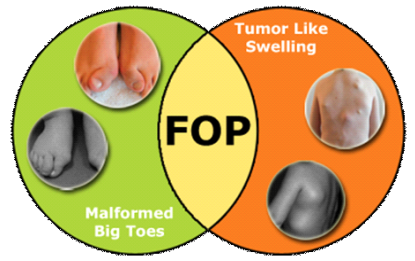
Severe fibrous dysplasia can cause:

- **Bone deformity or fracture :** The weakened area of an affected bone can cause the bone to bend. These weakened bones also are more likely to fracture.
- **Vision and hearing loss :** The nerves to your eyes and ears may be surrounded by affected bone. Severe deformity of facial bones can lead to loss of vision and hearing, but it's a rare complication.
- **Arthritis :** If leg and pelvic bones are deformed, arthritis may form in the joints of those bones.
- **Cancer :** Rarely, an affected area of bone can become cancerous. This rare complication usually only affects people who have had prior radiation therapy.

Diagnosis:

In most cases, an accurate diagnosis of fibrodysplasiaossificansprogressiva (FOP) can be made based on a patient's characteristic malformation of the big toe, in addition to rapidly changing swellings on the head, neck or back. Outbreaks may be measurable clinically by elevated levels of alkaline phosphatase and bone-specific alkaline phosphatase.

Due to a lack of knowledge of FOP among doctors, the rate of misdiagnosis of the disease is estimated at 80 percent or higher. These errors in diagnosing FOP have caused pain and suffering for FOP patients and their families worldwide. For instance, misdiagnosis has led to unnecessary invasive procedures, such as biopsies, as well as permanent complications from medical interventions, including loss of mobility.



Treatment:

Unfortunately, there is no effective treatment for fibrodysplasiaossificansprogressiva (FOP). Surgery is not an option for removing the excess bones because surgery often results in more bone formation. And these new bones don't disappear themselves.

Fibrodysplasia ossificans progressiva (FOP) should be diagnosed during the neonatal period. Early treatment of fibrodysplasia ossificans progressiva helps avoid the factors of aggravation, slow the progression of the disease, and provide the children with improved quality of life. No effective medical therapy is known for fibrodysplasia ossificans progressiva; bisphosphonates and corticosteroids are only beneficial during the flares. Gene therapy may hold promise in fibrodysplasia ossificans progressiva treatment.

Systemic steroids are sometimes used for acute flare-ups. Iontophoresis with steroids or acetic acid may improve diminished range of motion.

The good news is that researchers are investigating FOP and new treatments. For example, a drug is being developed that may help to control bone growth. Medications also are available to help relieve symptoms of FOP, such as pain and inflammation.

Surgical care:

Patients with fibrodysplasia ossificans, a rare disorder, may require oral surgical and anesthetic procedures to control oral pain. The importance of a minimally invasive surgical technique and appropriate anesthetic management has been stressed.

The experience using general anesthesia has been favorable, with awake nasal fiberoptic intubation evaluated as desirable for airway management.

FDA APPROVED NEW DRUGS FOR THE MONTH OF JUNE 2018

Category	Drug Name	Dose	Therapeutic indication	Company	Approved Date
Topical	Qbrexza (glycopyrronium)	2.4% (packaged in single-use individual pouch)	Hyperhidrosis	Dermira, Inc.	June 29, 2018
Melanoma	Braftovi (encorafenib)	Capsules 50mg 75mg	Melanoma - Metastatic	Array Bio-Pharma Inc.	June 27, 2018
Melanoma	Mektovi (binimetinib)	Tablet 15mg	Melanoma - Metastatic	Array BioPharma Inc.	June 27, 2018
Urinary Tract Infections	Zemdri (plazomicin)	500mg/10mL (50mg/mL)	Urinary Tract Infection	Achaogen, Inc.	June 25, 2018
Seizures	Epidiolex (cannabidiol)		Lennox-Gastaut Syndrome, Dravet Syndrome	GW Pharmaceuticals plc	June 25, 2018