What is IBMPFD?

IBMPFD is an adult onset, genetic disease that affects the muscles, bones, and brain. IBMPFD stands for Inclusion Body Myopathy associated with Paget's Disease of Bone and Frontotemporal Dementia. IBMPFD is an autosomal dominant disease caused by a mutation of the *Valosin Containing Protein* (*VCP* or p97) gene.

Some patients have familial Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig's disease) or Parkinson's, which has been shown to be caused by the pathogenic variant in *VCP*. Given the interaction with these other genetic diseases, IBMPFD may also be known as *VCP* Disease.

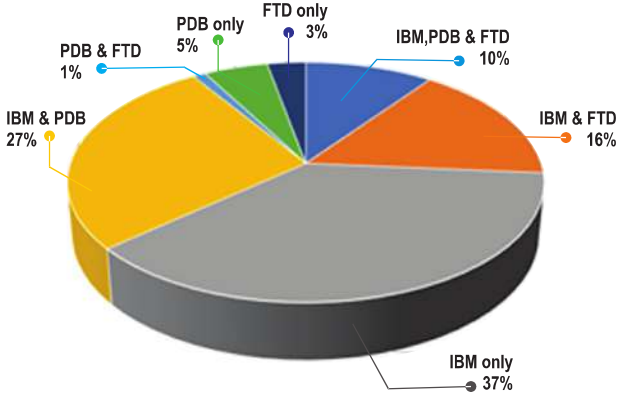
PENETRANCE BY PHENOTYPE

IBMPFD may manifest in any combination of the below three primary disorders. The below graph shows the percentage of known patients with each disorder combination.

IBM : Inclusion Body Myopathy

PDB : Paget's Disease of the Bone

FTD : Frontotemporal Dementia



Together We Can
Make a Difference

PATIENT SUPPORT

Cure VCP Disease, Inc. is a non-profit, patient advocacy group that was formed to drive efforts to treat and cure diseases related to mutations of the *Valosin Containing Protein (VCP)* gene. Our main goal is to bring patients and doctors together so we can help one another and drive research towards a treatment and cure.

We are also trying to bring awareness to the medical community about our rare disease. Currently, there are less than 500 people diagnosed worldwide.

CURE VCP DISEASE, INC.

We offer resources and support to patients, caregivers and doctors.

CONNECT WITH US

IBMPFD Facebook page | www.IBMPFD.com

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RARE FOUNDATION ALLIANCE MEMBER

Do you have a family history of ADULT ONSET

Muscle Weakness?
Dementia?
Bone Disease?
ALS?
Parkinson's?
IBM?

You may consider talking to your doctor about a mutation of the *VCP* gene called

IBMPFD

Inclusion Body Myopathy associated with Paget's Disease of Bone and Frontotemporal Dementia.

CURE VCP DISEASE, INC.

www.curevcp.org

Strength through numbers will help US find a CURE!

SYMPTOMS

A patient with *VCP* Disease / IBMPFD may have any combination of the following conditions:

Inclusion Body Myopathy (IBM): The first symptom of *VCP* Disease is often muscle weakness (myopathy). Typically, patients first notice difficulty raising arms, losing balance, or difficulty climbing stairs. As the disorder progresses, weakness develops in the other muscles in the arms, legs, and core muscles. Muscle weakness can also affect the respiratory and heart (cardiac) muscles. *VCP* Disease may initially be mis-diagnosed as limb-girdle myopathy. Symptoms may be first noticed in an individual's 20's to late 30's. Myopathy occurs in approximately 90% of affected individuals.

Paget's Disease of Bone (PDB): Bone pain, particularly in the hips and spine, is usually the major symptom of Paget's disease. Paget's most often affects bones of the hips, spine, skull, and pelvis. Paget's disease affects half of the individuals with *VCP* Disease and typically is first noticed in a person's 30's or 40's, which is a much earlier age than those without *VCP* Disease.

Frontotemporal Dementia (FTD): The brain is affected in about one-third of people with *VCP* Disease. FTD may change personality, behavior, language, and speech; memory is preserved. FTD typically presents in a person's mid-50's.

Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig's disease): ALS occurs in approximately 10% or more of individuals with *VCP* Disease. Symptoms include increased muscle tone, weakness, muscle wasting, muscle cramps, and difficulty swallowing and speaking.

RESEARCH

The goal of the CoRDS registry is to connect as many patients and researchers as possible to help advance treatments and cures for rare diseases.

If you have been diagnosed with *VCP* Disease / IBMPFD, please help us by completing the patient registry through CoRDS.

Go to our website

www.curevcp.org

to access the CoRDS Registry.

The CoRDS registry is free for patients to enroll and for researchers to access.

Please elect for researchers to have access to your registry, as that will greatly assist in the drive for a cure, especially when drug trials are recruiting patients.

CoRDS Registry
Coordination of Rare Diseases at Sanford

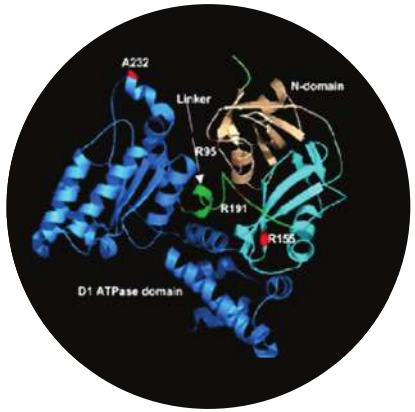
DIAGNOSIS

The only way of diagnosing *VCP* Disease / IBMPFD is through genetic testing. Consult with your doctor about testing for mutations of the *VCP* gene.

For information about genetic testing, our two medical advisors are available as resources:

Dr. Virginia Kimonis
Medical Geneticist
at University of California, Irvine
vkimonis@uci.edu

Dr. Conrad Weihl
Neuromuscular Specialist
at Washington University
weihlc@wustl.edu



TREATMENTS

There is an approved treatment for Paget's disease of bone, so early testing for this disorder is very important.

There are no known cures or treatments for the Inclusion Body Myopathy or Frontotemporal Dementia. Consult with your doctor about physical therapy, stretching, diet and mobility aids.

www.curevcp.org