

# BÖLÜM 47

## PEDİATRİK NÖROLOJİ HASTALARINDA KÖK HÜCRE UYGULAMALARI

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### GİRİŞ

Kök hücreler, klonojenik ve kendini yenileme yetenekleri olan ve çoklu hücre soylarına farklılaşma yeteneğine sahip hücreler olarak tanımlanır.<sup>1-4</sup> Kendi kendini yenileme (self-renewal), klon oluşturabilme (cloning), çoğalma (proliferasyon) ve farklılaşma (diferansiyasyon) başlıca özellikleridir.<sup>1-3</sup> İlk olarak hematopoetik sistemde tanımlanan kök hücreler, hem insan gelişiminin ilk aşamalarında (embriyolarda) hem de yetişkin hücrelerde bulunur.<sup>1-3</sup>

**Self renewal:** kök hücre literatürü, bir hücrenin replikasyon kapasitesini tanımlamak için 'ölümsüz', 'sınırsız', 'sürekli' gibi terimlerle doludur. Bu tür terimlerin kullanımından mümkün olduğu sürece kaçınılmalı veya dikkatli kullanılmalıdır. Açık bir ifadeyle, kök hücreler atalarına özdeş yavru hücreler üretebilir ve bu yetenek "self renewal" (kendini yenileme) olarak ifade edilir.<sup>4</sup>

Kök hücreler, insan vücudunun özelleşmiş hücreleridir ve organizmanın herhangi bir hücresine farklılaşabilir ve daha sınırlı potansiyele sahip yeni nesiller (farklılaşmış hücreler) üretebilirler.<sup>3,4</sup> Farklılaşmamış kök hücrelerin bölünerek belli bir fonksiyon gören hücreye

dönüşmesine **diferansiyasyon** denir.<sup>4,5</sup> Bu yetenek sayesinde kan, kas, karaciğer gibi özelleşmiş görev yapan organları oluşturabilirler ve bu özellik kök hücreleri eşsiz kılar.<sup>1-5</sup>

**Klonalite:** Klon oluşturma (klonalite). Genellikle kültürde bulunan bir hücre popülasyonunun nasıl türetildiğini tanımlayan bir özelliktir. Hücre hattını neyin oluşturduğu sorulduğunda klonalite çok önemli hale gelir. Klonal bir popülasyon, kendi kendini yenileme kapasitesine sahip bir kök hücre gibi tek bir hücreden üretilir.<sup>4</sup>

**Potens:** Bir hücrenin diğer hücrelere farklılaşma potansiyelidir. Totipotent, pluripotent, multipotent, oligopotent ve unipotent terimleri potens kavramının alt kırımlarıdır. Potansiyel sorunu, kök hücrenin yaygın olarak kabul edilen bir tanımına dahil edilmesi en zor parametredir.<sup>4</sup> Farklılaşmanın birkaç aşaması vardır. Gelişim potansiyeli farklılaşmanın her adımında azalır, bu da unipotent bir kök hücrenin pluripotent bir kök hücre kadar çok hücre tipine farklılaşamayacağı anlamına gelmektedir.<sup>3</sup>

**Totipotent** kök hücreler bölünebilir ve organizmanın tüm hücrelerine farklılaşabilme potansiyeline sahip ilk embriyonel kök hücre

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myastenia gravis (MG), glioblastoma gibi bazı beyin tümörlerinde, ataksi telenjipektazi (A-T), Friedreich ataksisi, Rett sendromu, Gaucher hastalığı, Lesch-Nyhan sendromu, Down sendromu, fragil X sendromu, tüberoz skleroz, Prader-Willi sendromu, Angelman sendromu, Alexander hastalığı (AxD), familial disotonomi, mitokondriyal miyopati, ensefalopati, laktik asidoz ve felç benzeri ataklar (MELAS), herediter spastik parapleji, girat atrofi, mikrosefali, subakut sklerozan panensefalit (SSPE), yaşa bağlı maküler dejenerasyon (AMD), maküler distrofi ve retinitis pigmentosa (RP) gibi retina dejenerasyonlarında ve şizofreni gibi çok sayıda nörolojik hastalıkta kök hücre tedavileri denenmektedir.<sup>115,150-161,171,178,179,181,182.</sup>

Bu çalışmalarının bir çoğunda sonuçlar umut vaad edicidir. Ancak SSPE'de MKH uygulaması ile ilgili ilk vaka raporunda fayda gözlenmediği bildirilmiştir.<sup>181</sup>

Bir diğer uygulama alanı prenatal kök hücre tedavisidir. Kalıtsal hastalıklarda kullanımı ile ilgili çalışmalar vardır. Ancak bu konu ile ilgili olarak ele alınması gereken çok sayıda etik sorun söz konusudur.<sup>128</sup>

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